VALUATIONLAB
FINANCIAL ANALYSIS

SANTHERA PHARMACEUTICALS

FOCUS AREA: NOVEL THERAPIES IN RARE NEUROMUSCULAR AND PEDIATRIC DISORDERS			
KEY DATA			SIX: SANN
MARKET CAPITALIZATION (CHF MN)	124	**SHARE PRICE ON 04 JUNE 2024**	9.8
ENTERPRISE VALUE (CHF MN)	122	**RISK-ADJUSTED NPV PER SHARE** (CHF) *	39
GROSS CASH (31 MARCH 2024) (CHF MN)	27	**UPSIDE/DOWNSIDE** (%)	300%
MONTHLY OPERATING EXPENSE (CHF MN)	4.4	**RISK PROFILE**	SPECULATIVE
CASH RUNWAY **	INTO 2025	**SUCCESS PROBABILITY LEAD PROJECT**	100%
BREAK-EVEN (YEAR)	H1 2026	**EMPLOYEES**	70
FOUNDED (YEAR)	2004	**LISTED** (YEAR)	2006
KEY PRODUCTS:	**STATUS**	**MAJOR SHAREHOLDERS:**	**(%)**
- AGAMREE (DUCHENNE MUSCULAR DYSTROPHY - DMD)	APPROVED IN US, EU & UK	- CATALYST PHARMACEUTICALS	11.2
- AGAMREE (BECKER MUSCULAR DYSTROPHY - BMD)	PHASE II POC	- IDORSIA	10.3
- AGAMREE (RARE PEDIATRIC DISEASES)	TBD	- EXECUTIVE MANAGEMENT & BOARD	0.0
		- FREE FLOAT	88.7
		- AVERAGE DAILY VOLUME (30-DAY)	21'722
UPCOMING CATALYSTS:	**DATE**	**ANALYST(S):**	BOB POOLER
- AGAMREE - UK LAUNCH	SUMMER 2024		BP@VALUATIONLAB.COM
- AGAMREE - COMMERCIALIZATION OTHER REGIONS BY PARTNERS	DURING 2024		+41 79 652 67 68
- AGAMREE - CHINA APPROVAL / LAUNCH FRANCE, ITALY, SPAIN	Q1 2025		

NOTE: * 17.7 MN SHARES USED FOR CALCULATION OF RISK-ADJUSTED NPV/SHARE, ASSUMING ADDITIONAL CHF 50 MN NEEDED TO REACH BREAKEVEN; ** EXCLUDING MATURING CONVERTIBLE BONDS IN AUGUST 2024

ESTIMATES AS OF 4 JUNE 2024 SOURCE: VALUATIONLAB ESTIMATES, SANTHERA PHARMACEUTICALS

On steroids - but better!

AGAMREE launch signals a new era for Santhera

Santhera is focused on developing and commercializing treatments for rare neuromuscular and pediatric disorders. Its key driver is vamorolone, branded AGAMREE. This first-in-class dissociative oral steroid has just been launched in the US and the EU (Germany, Austria, with other member states to follow in early 2025) for treating Duchenne muscular dystrophy (DMD), with the UK launch this summer. Santhera acquired the global rights to AGAMREE for all indications from Idorsia (10.3% stakeholder) and ReveraGen, with the potential to monetize geographical rights and non-DMD indications. AGAMREE targets a multibillion-dollar market with high unmet medical need. The launch of AGAMREE in DMD, with its first revenues in 2024, signals a new era for Santhera. The Catalyst (11.2% stakeholder) license agreement for AGAMREE in North America, valued at up to USD 231 mn, together with the first revenues of AGAMREE, milestone payments, the full divestment of Raxone to Chiesi, and the termination of lonodelestat development, provides a cash runway into 2025 (excluding maturing convertible bonds). Santhera guides for AGAMREE sales of more than EUR 150 mn in Europe in DMD alone within the next five years and to start breaking even on a cash basis by H1 2026. We derive a sum-of-parts risk-adjusted (r)NPV of CHF 39 per share. We qualify Santhera as High Risk (from Speculative) following the approval of AGAMREE in DMD in the major markets.

Key catalysts:

1) **AGAMREE UK launch in DMD (summer 2024):** Shortly after the completion of pricing reviews by NICE (National Institute for Health and Care Excellence).
2) **AGAMREE partnerships (during 2024):** Santhera expects to sign commercialization partners for AGAMREE during 2024 outside its own territories, North America (Catalyst), and China (Sperogenix), with a priority for European distributors to support the European rollout.
3) **AGAMREE China approval; launch in France, Italy, Spain, Benelux (Q1 2025):** Sperogenix, Santhera's development and commercialization partner for China, expects approval in China following the granted priority review. Around the same time, Santhera expects to launch AGAMREE in France, Italy, Spain, and the Benelux countries to treat DMD with its specialist sales force.

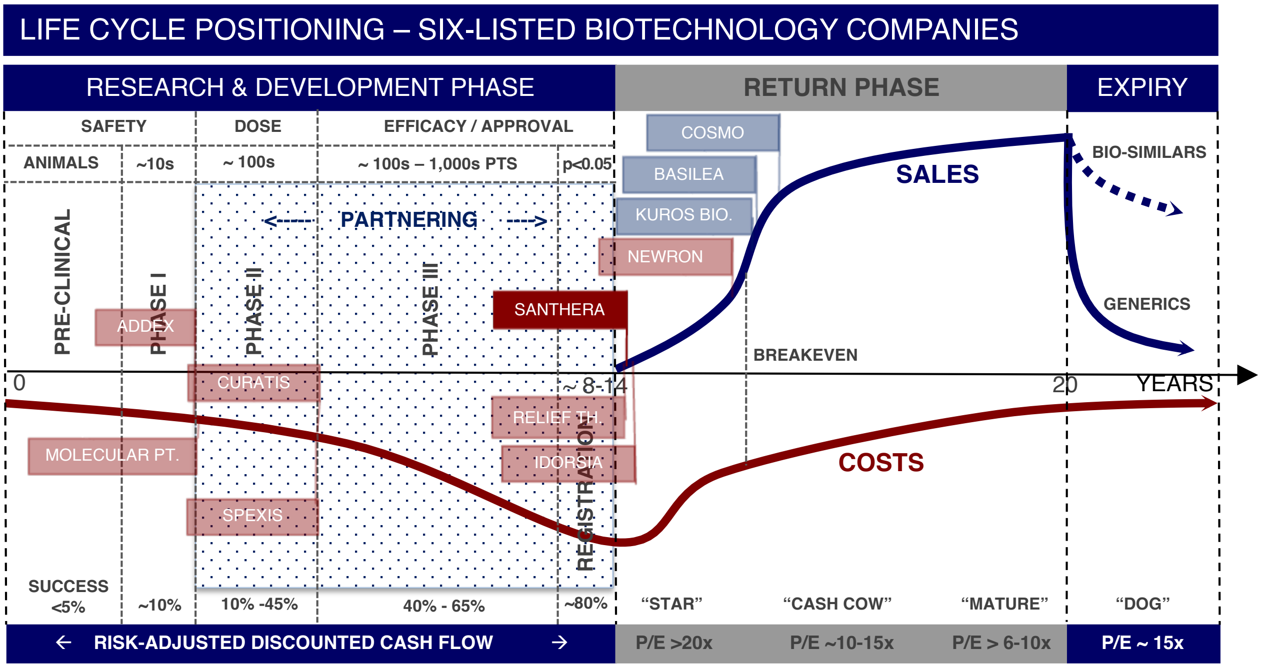
Investment Case, Strategy & Cash

Investment case in a nutshell

The unprecedented three full approvals of AGAMREE in DMD in the US, EU, and UK within a space of three months signals a new era for Santhera. 2024 is set to be a transformational year with the first AGAMREE revenues. The first launch globally started in 2024 in Germany in January, followed by Austria in February, with more EU countries to follow in early 2025. The US launch started in March, with the UK launch to follow this summer. AGAMREE has the potential to become a foundational DMD treatment, replacing current steroid therapy hampered by side effects, and can be used alone or in combination with approved DMD drugs and new gene therapies in all DMD patients. The Catalyst (AGAMREE North America rights) and Chiesi (full divestment of Raxone) agreements in 2023 extended the cash runway into 2025 (excluding maturing convertible bonds - CBs), significantly strengthened the balance sheet, repaid the Highbridge debt, and paid for third-party AGAMREE milestones in North America. Santhera guides AGAMREE European sales in DMD alone of more than EUR 150 mn, reaching breakeven in H1 2026. Based on our bottom-up forecasts for AGAMREE in DMD, with global CHF 750+ mn peak sales (incl. partner sales), ample patent life, and market exclusivity, we calculate a risk-adjusted (r)NPV of CHF 696 mn or CHF 39 per share, providing 300% equity upside. We conservatively allow for a 40% dilution for an additional CHF 50 mn capital to repay the maturing CBs in August and support further AGAMREE co-development with Catalyst in new rare pediatric indications. Santhera will prioritize debt financing and monetization of royalties over equity options, which should lead to far less dilution than we forecast at the current low share price.

Life Cycle Positioning – Speculative

We qualify Santhera's risk profile as High Risk (from Speculative) following the approval and launch of AGAMREE. In our view, the recent launches and emerging revenues of AGAMREE in DMD will be transformational, leading to a substantial re-rating and breakeven targeted for H1 2026 (for our Risk Qualification, see Important Disclosures).



SOURCE: VALUATIONLAB

Swiss specialty pharma focused on rare neuromuscular & pulmonary diseases
Santhera Pharmaceuticals is a Swiss specialty pharmaceutical company focused on developing and commercializing prescription drugs to treat rare neuromuscular and pediatric diseases. With over 200 such diseases, this area of high unmet medical need includes many orphan (rare) and niche indications, typically with no or few effective therapies. These conditions are usually genetic (inherited), treated by a few physicians (implying lower marketing costs), and typically command premium pricing. Development is spurred through special "orphan drug" programs with a more focused development timeframe and prolonged market exclusivity protection. Santhera Pharmaceuticals was founded in September 2004 through a merger between Germany-based Graffinity Pharmaceuticals AG and Swiss-based MyoContract AG, both privately held.

Its lead drug is vamorolone, branded AGAMREE. It received full approval to treat Duchenne muscular dystrophy (DMD), a rare genetic disorder that causes progressive muscle weakness and degeneration predominantly in boys and is being rolled out in the US, EU, and UK, with estimated global peak sales of CHF 750+ mn (including partner sales). In September 2020, Santhera acquired the global rights to AGAMREE for all indications from Idorsia and originator ReveraGen.

In November 2006, Santhera was listed on the SIX Swiss Stock Exchange through a successful IPO (initial public offering). Its global headquarters is based in Pratteln (near Basel), Switzerland. Santhera has 70 employees globally but expects to increase that number by up to 60 incremental employees over the next two years to expand its specialist sales force to successfully rollout AGAMREE in the top-5 Europe (Germany, UK, France, Italy, Spain), the Benelux (Belgium, Netherlands, Luxemburg), Switzerland and Austria.

Strategy to create value by becoming a full-fledged rare disease specialty company
Santhera's strategy is to create value by building a comprehensive portfolio of compounds in rare diseases through in-licensing, subsequently developing these compounds up to market approval through their development capabilities and expertise in rare diseases, and ultimately commercializing these compounds through their own small specialist sales force in key markets to retain and maximize value.

Santhera is engaged in the development and commercialization of drugs for rare diseases with a special focus on:

Neuromuscular diseases: treatments for diseases that impair the functioning of the muscles, including the muscles directly or indirectly affecting the nerves that control muscles or the communication between muscles and nerves; Santhera's key neuromuscular drug includes:

- **AGAMREE** (vamorolone) for the treatment of DMD patients with early-to-mid-stage disease; we forecast global peak sales of CHF 750+ mn (including partner sales) in DMD alone; fully approved within a space of three months in the US, EU, and the UK based on the positive results of the pivotal "VISION-DMD" trial with similar efficacy as in-market steroids but with a superior safety and tolerability profile lacking typical "steroid" side effects such as stunted growth, bone fractures, and behavioral disorders; AGAMREE is the only DMD drug to receive full regulatory approval for DMD in the US, EU, and UK, with the exception of PTC Therapeutics' mainstay steroid Emflaza (deflazacort) in the US, which it is expected to replace, all other DMD drugs received only conditional/accelerated approval; in January 2024, the European launch started in Germany by Santhera's own specialist sales force with the European country-by-country rollout to continue into early 2025 with additional distribution partners to be signed on; the US launch by Catalyst Pharmaceuticals, the commercialization partner for North America, started in March 2024; Santhera is still eligible for up to USD 105 mn sales milestones and tiered up to low-teen percentage royalties on net sales in North America (with Catalyst to pay third-party royalty obligations in North America); Sperogenix, the commercialization partner for the Greater China region, filed in China with launch expected in Q1 2025 thanks to granted Priority Review, with up to USD 104 mn additional milestones and double-digit royalties on net sales; potential for out-licensing deals in geographies outside the Santhera territories (top-5 Europe, Benelux, Austria, Switzerland), North America (Catalyst), and Greater China (Sperogenix) or for non-DMD indications, including major inflammatory diseases such as inflammatory bowel disease (IBD), asthma or chronic obstructive pulmonary disease (COPD), juvenile dermatomyositis, (pediatric) ulcerative colitis, among others, could add to the cash position; Idorsia has 10.3% stake in Santhera, while Catalyst has an 11.2% stake.

Fully focused on AGAMREE by completing the exit of non-core products

Santhera strategically decided to fully concentrate its efforts and resources on AGAMREE in DMD and, together with Catalyst, maximize the value in potential additional indications and broaden its therapeutic reach. It has completed the exit of non-core products:

Raxone fully divested to Chiesi Group to free up resources in 2023

In July 2023, Santhera's idebenone (branded "Raxone") business, including Raxone in Leber's hereditary optic neuropathy (LHON), was fully divested to the Italian Chiesi Group (Chiesi). In 2019, Chiesi acquired global rights, except for North America and France. Under the terms of the new agreement, Chiesi assumes the French reimbursement liability of EUR 25.3 mn (due in 2024/2025) related to Raxone in LHON, significantly reducing near-term financial obligations and strengthening Santhera's balance sheet. Santhera retained rights to participate in a potential approval of Raxone in LHON in the lucrative US market through single-digit variable payments on net sales or milestone payments of up to USD 10 mn. Santhera is also eligible for additional milestone payments in a similar order of magnitude and high single-digit variable payments on net sales in potential non-ophthalmological indications for Raxone. We have conservatively excluded any revenues for Raxone in our forecasts.

Lonodelestat rights returned to Spexis in early 2024

The development of lonodelestat in rare lung disorders has been paused since late 2022 due to limited financial resources, with Santhera prioritizing the development and commercialization of AGAMREE. Santhera acquired the global rights for lonodelestat from Spexis (previously Polyphor) in 2018. After a thorough analysis of the changing therapeutic landscape in cystic fibrosis and other lung disorders, the company did not see sufficient potential to justify continuing the development of lonodelestat. In early 2024, the license agreement has been terminated, and the compound will be returned to Spexis.

Q1 2024 trading update - Initial AGAMREE sales uptake surpasses expectations
AGAMREE is off to a flying start. Santhera reported Q1 2024 revenue of CHF 4.7 mn largely from initial product sales of AGAMREE in Germany and Austria of CHF 2.1 mn and milestone payments from Sperogenix on the filing in China and supply of AGAMREE to partners of CHF 2.6 mn. The reception in Germany and Austria has been very positive, with strong demand and proactive requests from patients and caretakers. Within only a few months of availability, AGAMREE has been prescribed to around 150 patients (~5% of total DMD patients) in Germany and Austria. In the US, Catalyst reported that the early AGAMREE launch indicators highlight a promising product uptake surpassing initial expectations. In Q1 2024, net product revenues of AGAMREE amounted to USD 1.2 mn in just the first two weeks of commercial availability in the US.

Santhera's key priorities for the next 12-18 months include:

- **EU rollout of AGAMREE:** On 15 January 2024, the first launch of AGAMREE in Santhera territories started in Germany, followed by Austria in February. After clearing pricing and reimbursement, the UK launch is expected this summer, followed by France, Italy, Spain, and the Benelux in early 2025. Establishing competitive pricing and reimbursement in each EU member state and the UK is crucial for sales uptake.

- **Build out European commercial infrastructure:** establish a specialist sales infrastructure for the top-5 Europe (Germany, UK, France, Italy, Spain), Benelux (Belgium, Netherlands, Luxemburg), Austria, and Switzerland, and add commercialization partners or distributors for the remaining EU countries.

- **US launch of AGAMREE:** occurred in March 2024 by Catalyst, Santhera's commercialization partner for North America (US, Canada, Mexico). It is dispensed throughout the US through a specialty pharmacy network supported by a comprehensive Patient Assistance Program to enhance patient and sales uptake.

- **New AGAMREE indications:** in collaboration with Catalyst, Santhera is preparing clinical work to extend AGAMREE's therapeutic benefits to additional indications with a high unmet medical need with a special focus on rare pediatric disorders.

- **Broaden product pipeline offering:**
  - In-license new development projects in rare diseases
  - In-license new products to leverage its European sales force infrastructure

Cash runway into Q1 2025 – Breakeven on a cash basis guided for H1 2026
The substantial infusion of funds brought by the Catalyst license agreement for the rights of AGAMREE in North America valued at up to USD 231 mn plus royalties, substantially reduced debt by the repayment of the Highbridge loan, while the full divestment of Raxone to Chiesi, reduced near-term obligations, extending Santhera's cash reach into Q1 2025 (excluding maturing convertible bonds). This provides a strong financial foundation for future activities, including advancing European commercialization and AGAMREE in new indications. Further capital of around CHF 50 mn may be needed to repay the outstanding convertible bonds (CHF 24.6 mn) due in August 2024 and to advance AGAMREE in new indications together with Catalyst and extend the cash runway to breakeven. Santhera continues to evaluate options for additional financing and will prioritize debt financing and

monetization of royalties over equity options, limiting potential share dilution at the current relatively low valuation.

Within the next five years, Santhera estimates it will achieve annual sales of AGAMREE more than EUR 150 mn (>CHF 147 mn) in Europe in DMD alone, with additional revenue from milestones and royalties from its partners in the US and China. Beyond that, the company is seeking to widen geographic access to AGAMREE through additional distribution partnerships in yet-uncovered regions. Santhera expects to start breaking even on a cash basis by H1 2026.

Valuation Overview

Sum-of-parts risk-adjusted (r)NPV points to a fair value of CHF 39 per share
We derive a sum-of-parts rNPV of CHF 39 per share for Santhera, with estimated cash and cash equivalents (31 March 2024) of CHF 2 per share and overhead expenses of CHF 10 per share (including the repayment of the maturing convertible bonds in August 2024), assuming a WACC of 10% (consisting of a specific risk of 9% (= market premium risk of 6% multiplied by a beta of 1.5) and a risk-free rate of 1%).

SUM OF PARTS

PRODUCT	INDICATION	PEAK SALES (CHF MN)	LAUNCH YEAR	UNADJUSTED NPV/SHARE (CHF)	SUCCESS PROBABILITY	RNPV/SHARE (CHF)	PERCENTAGE OF TOTAL
AGAMREE (VAMOROLONE)	DMD * (STEROID REPLACEMENT)	766	2024	48	100%	**48**	97%
AGAMREE (VAMOROLONE)	BECKER MUSCULAR DYSTROPHY (BMD)	TBD	TBD	TBD		**TBD**	
CASH & CASH EQUIVALENTS (31 MARCH 2024)		27		2		**2**	3%
TOTAL ASSETS				50		**50**	**100%**
OVERHEAD EXPENSES (INCL. REPAYMENT OF CONVERTIBLE BONDS DUE AUG 2024)				-10		**-10**	
NPV/SHARE (CHF)				39		**39**	
SHARE PRICE ON JUNE 04, 2024						9.8	
PERCENTAGE UPSIDE / (DOWNSIDE)						300%	

NOTE: * 17.7 MN SHARES USED FOR CALCULATION OF RISK-ADJUSTED NPV/SHARE, ASSUMING ADDITIONAL CHF 50 MN NEEDED TO REACH BREAKEVEN H1 2026; * DMD = DUCHENNE MUSCULAR DYSTROPHY
ESTIMATES AS OF 4 JUNE 2024 SOURCE: VALUATIONLAB ESTIMATES

Santhera's key driver:

AGAMREE in DMD (all patients) – rNPV of CHF 48/share
AGAMREE targets DMD patients with early-to-mid-stage disease to preserve muscle function, effectively replacing mainstay steroid therapy, such as prednisone and deflazacort (branded "Emflaza" in the US by PTC Therapeutics with USD 255 mn sales in 2023), irrespective of the underlying genetic mutation. We forecast global peak sales of CHF 750+ mn (including partner sales) for AGAMREE in DMD alone. Within the next 5 years, Santhera targets peak sales of >EUR 150 mn in its European territories, including the top-5 Europe (Germany, UK, France, Italy, Spain), the Benelux (Belgium, Netherlands, Luxemburg) and Switzerland and Austria, where it will commercialize AGAMREE through an own specialist sales force. The first launch occurred in Germany in January 2024, followed by Austria in February and the UK this summer. France, Italy, Spain, and the Benelux countries will follow in early 2025. Outside its own key EU markets, the company will seek partners.

At the end of March 2024 AGAMREE was launched in the US by Catalyst. Early launch indicators highlight a promising uptake, surpassing initial expectations with Q1 2024 revenue of USD 1.2 mn in its first two weeks on the market. Santhera is eligible for up to USD 105 mn in sales milestones with tiered up to low-teen percentage sales royalties, with Catalyst paying for the royalty obligations (tiered single-digit to low double-digit percentage royalties) in North America to Idorsia and ReveraGen.

In the Greater China Region, Sperogenix will commercialize AGAMREE in return for up to USD 124 mn milestones (including additional indications) and double-digit royalties on net sales. Approval in China is expected in Q1 2025. We calculate an NPV of CHF 48/share for AGAMREE in DMD alone.

We have conservatively not accounted for additional indications for AGAMREE, such as Becker muscular dystrophy (BMD), due to the lack of sufficient proof-of-concept yet.

Sensitivities that can influence our valuation

Funding risk: The Catalyst agreement with a value of up to USD 231 mn with significant upfront payments, together with the full divestment of Raxone to Chiesi in 2023, is guided to provide a cash runway into Q1 2025 (excluding maturing convertible bonds). Santhera continues to evaluate the monetization of pipeline assets such as AGAMREE in DMD in certain geographies outside its own territories, North America (Catalyst), Greater China region (Sperogenix), and in large non-DMD indications such as asthma, COPD, or ulcerative colitis. Additional capital of CHF 50 mn may be needed for the repayment of the CBs maturing in August 2024 and the co-development of AGAMREE in new indications. Upfront milestone payments from new AGAMREE partnerships could lower the additional capital needed, with Santhera preferring debt financing and monetization of royalties over equity options.

Development risk: AGAMREE has been successfully developed in its main indication, DMD, and is currently being rolled out. We have conservatively not included any forecasts for other potential indications such as BMD or other indications where AGAMREE could be effective in.

Approval risk: AGAMREE has been fully approved for treating DMD in the major markets, including the US (October 2023), the EU (December 2023), and the UK (January 2024), with China expected to follow in 2025. In contrast, all other DMD drugs (except for PTC Therapeutics' steroid Emflaza) have only received conditional/accelerated approval from the EMA or the FDA with additional clinical data requirements to establish a positive benefit/risk and maintain their marketing authorization. AGAMREE has been launched in the US, in the first EU countries, with the UK launch planned for this summer.

Pricing and reimbursement risk: Following approval of AGAMREE, pricing and reimbursement have to be negotiated. In the US, pricing is quite straightforward, while in the EU, this has to be negotiated individually in each member state. Pricing could be lower, and negotiations could take longer than forecast. Orphan drugs such as AGAMREE typically command a high price. Santhera plans to price its drugs "attractively" compared to competitors thanks to lower COGS, enhancing reimbursement and market penetration.

Commercialization risk: Catalyst has an established sales infrastructure in North America with sufficient marketing muscle to successfully commercialize AGAMREE in DMD. Santhera will commercialize AGAMREE through its own specialist sales force in the top-5 Europe (Germany, UK, France, Italy, Spain), the Benelux (Belgium, Netherlands, Luxemburg), Switzerland, and Austria to maximize long-term profitability. Sperogenix acquired the commercialization rights for the Greater China region. In the ROW, Santhera plans to seek commercialization partners in return for upfront, regulatory, and sales milestones and royalties on sales like the Sperogenix agreement. Delays could occur in the buildup and rollout of AGAMREE. Santhera largely depends on its partners' sales efforts and marketing muscle to commercialize AGAMREE, where delays can occur, and actual terms could differ from our estimates.

Catalysts

CATALYST TIMELINES					
TIME LINE	PRODUCT	INDICATION	WHAT	COMMENT	IMPACT ON RNPV/SHARE
2024					
DURING	AGAMREE (VAMOROLONE)	DMD* & NON-DMD* INDICATIONS	PARTNERING OPPORTUNITIES OUTSIDE NORTH AMERICA, CHINA, EUROPE	DISTRIBUTION PARTNERS OR LICENSING PARTNERING OF AGAMREE OUTSIDE NORTH AMERICA (CATALYST), GREATER CHINA (SPEROGENIX) AND EUROPE (SANTHERA TERRITORIES) FOR SUBSTANTIAL UPFRONT, REGULATORY AND SALES MILESTONES AND ROYALTIES ON SALES; POTENTIAL TO PROVIDE NON-DILUTIVE FUNDING	
12 JAN	AGAMREE	DMD*	UK APPROVAL	UK APPROVAL FOR AGAMREE AS A TREATMENT FOR DMD; LAUNCH BY SANTHERA SPECIALIST SALES FORCE IN SUMMER 2024	
15 JAN	AGAMREE	DMD*	FIRST LAUNCH IN GERMANY	FIRST COMMERCIAL LAUNCH OF AGAMREE GLOBALLY IN DMD OCCURRED IN GERMANY; AGAMREE WILL BE LAUNCHED THROUGH ITS OWN SPECIALIST SALES FORCE IN A STAGED ROLL-OUT IN THE TOP 5 EUROPE (GERMANY, UK, FRANCE, ITALY, SPAIN), BENELUX (BELGIUM, NETHERLANDS, LUXEMBURG), SWITZERLAND AND AUSTRIA; ADDITIONAL REVENUES FROM THE REMAINING EU COUNTRIES WILL COME FROM DISTRIBUTION PARTNERS	
19 JAN			END CATALYST LOCK-UP	END OF CATALYST 6-MONTH LOCK-UP AFTER CLOSING OF THE EXCLUSIVE LICENSE AGREEMENT OF AGAMREE FOR NORTH AMERICA	
14 MAR	AGAMREE	DMD*	US LAUNCH	NORTH AMERICA COMMERCIALIZATION PARTNER CATALYST PHARMACEUTICALS LAUNCHED AGAMREE IN THE LUCRATIVE US MARKET THROUGH ITS RARE DISEASE SPECIALIST SALES FORCE; A COMPREHENSIVE PATIENT ASSISTANCE PROGRAM IS AVAILABE UPON LAUNCH TO ENHANCE PATIENT ENROLLMENT AND SALES UPTAKE	
27 MAR	AGAMREE	DMD*	NDA & PRIORITY REVIEW GRANTED IN CHINA	THE NDA (NEW DRUG APPLICATION) FOR AGAMREE IN DMD WAS ACCEPTED AND PRIORITY REVIEW WAS GRANTED BY CHINA'S NMPA (NATIONAL MEDICAL PRODUCTS ADMINISTRATION); APPROVAL IN CHINA IS EXPECTED IN Q1 2025 (TRIGGERS HIGH SINGLE DIGIT MILLION US DOLLAR MILESTONE FROM SPEROGENIX)	
25 APR			FY 2023 PRELIMINARY RESULTS & Q1 2024 TRADING UPDATE	**FY 2023 RESULTS:** REVENUE FROM CONTRACTS WITH CUSTOMERS CHF 103.4 MN (2023: CHF 7.5 MN) BOOSTED BY CATALYST DEAL, OPERATING RESULT CHF 68.8 MN (2022: CHF -52.0 MN), NET RESULT CHF 54.8 MN (2022: -71.1 MN) BOOSTED BY CATALYST DEAL AND LOWER OPERATING EXPENSES OFFSETTING LOWER NET FINANCIAL INCOME; CASH AND CASH EQUIVALENTS CHF 30.4 MN (31 DECEMBER 2023), CASH RUNWAY INTO 2025 (EXCLUDING CONVERTIBLE BOND MATURITY IN AUGUST 2024) **FY 2024 GUIDANCE:** CASH NET OUTFLOW FROM OPERATIONS APPROXIMATELY CHF 2.5 MN PER MONTH (FROM APPROXIMATELY CHF 4 MN/MONTH EXCLUDING ONE-OFF LICENSING INCOME) **Q1 2024 TRADING UPDATE:** REVENUE CHF 4.7 MN FROM AGAMREE SALES IN GERMANY AND AUSTRIA (CHF 2.1 MN) AND MILESTONE PAYMENT NDA FILING CHINA AND SUPPLY OF PRODUCT TO PARTNERS (CHF 2.6 MN); CASH AND CASH EQUIVALENTS CHF 26.8 MN (31 MARCH 2024)	
28 MAY			FY 2023 ANNUAL REPORT & RESULTS	PUBLICATION OF THE FY 2023 ANNUAL REPORT AND AUDITED RESULTS - NO MAJOR CHANGES TO THE FY 2023 PRELIMINARY UNAUDITED RESULTS REPORTED ON 25 APRIL (SEE ABOVE)	
18 JUN			AGM	ANNUAL GENERAL MEETING (AGM)	
H2	AGAMREE	RARE PEDIATRIC DISEASES	NEW INDICATIONS	MORE INFORMATION IS EXPECTED TO BE RELEASED ON NEW RARE PEDIATRIC INDICATIONS FOR AGAMREE - CATALYST AND SANTHERA ARE WORKING CLOSELY TOGETHER TO JOINTLY DEVELOP NEW INDICATIONS TO BROADEN THE USE OF AGAMREE BEYOND DMD IN RARE PEDIATRIC DISEASES	
AUG			2021/2024 CONVERTIBLE BONDS	THE 2021/2024 CONVERTIBLE BONDS WITH AN AGGREGATE PRINCIPAL AMOUNT OF CHF 30 MN ARE EXPECTED TO BE FULLY REPAID IN AUGUST 2024	

ESTIMATES AS OF 4 JUNE 2024 — SOURCE: VALUATIONLAB ESTIMATES

Technology & Pipeline

Aim to develop re-profiled compounds in rare diseases
Santhera does not have its own discovery or research platform but has vast experience in the clinical development of rare diseases. Moreover, the company was one of the first specialty pharmaceutical companies to see substantial value in developing and repositioning existing compounds neglected by “Big Pharma” in rare (orphan) diseases. Consequently, all compounds have been licensed in or acquired.

Orphan disease programs provide attractive incentives for Santhera:
Although individual orphan diseases may be classified as uncommon, collectively, they affect a large portion of the population and healthcare expenditure. The US and EU orphan disease programs have been developed to provide pharmaceutical companies a strong incentive to pursue and develop orphan prescription drugs for these rare disorders, providing additional years of market exclusivity. AGAMREE enjoys these market exclusivities next to ample patent protection.

Key advantages to developing drugs in orphan indications include:

- Orphan drug disease (ODD) market exclusivity of 7 years (US) or 10 years (EU) starting from the first day of approval provides sufficient time for an attractive return.
- Pediatric market exclusivity provides additional 2-year (EU) and 6-month (US) market exclusivity.
- There are often no approved drugs for these indications, or only a few.
- Effective treatments can command a relatively high price, resulting in high margins.
- Competition is often limited to a few players.
- Specialists can be addressed by a relatively small specialist sales force.
- Conditional (EU)/Accelerated (US) early approval can be granted in the absence of robust trial data.

However, there are also considerable hurdles, including:

- A very low number of patients to conduct clinical trials – lack of robust clinical data, slow enrollment, trial delays.
- A lack of widespread expertise in clinical centers.
- Insufficient understanding of the history or mechanism of the disease.
- Absence of a clear regulatory pathway on how to set up the pivotal clinical trial, including what the right endpoints should be.
- The small number of experts who conduct the trials are often banned from advisory panels.

Portfolio review - Fully focused on AGAMREE to expand and broaden its potential
Santhera’s therapeutic focus is on rare neuromuscular and pediatric disorders. The company has strategically decided to concentrate all its efforts on vamorolone (branded AGAMREE). AGAMREE is a “pipeline in a product” being rolled out in its first approved indication, DMD, with a huge potential in other (rare) inflammatory diseases. Together with Catalyst, Santhera wants to extend its potential in additional indications and broaden its therapeutic reach, starting with rare pediatric diseases. Once AGAMREE has been developed in a more convenient tablet formulation, other large inflammatory diseases where corticosteroids are used could be pursued.

In 2023, the entire idebenone business—previously its cornerstone therapy—including Raxone in Leber's hereditary optic neuropathy (LHON) was divested to Chiesi, which had already held exclusive rights globally, except for North America and France, since 2019. As a result, Chiesi assumed Santhera's French reimbursement liability of EUR 25.3 mn, significantly reducing its near-term financial obligations.

In early 2024, Santhera also terminated the development activities and license agreement for lonodelestat and will return the compound to its originator, Spexis (previously Polyphor). Global rights to lonodelestat were acquired in 2018. Clinical development in the target indication of cystic fibrosis has been paused since 2022 due to limited resources and prioritizing the development and commercialization of AGAMREE in DMD. New and effective treatment options for cystic fibrosis, such as CFTR modulators, have also contributed to the decision to terminate development.

AGAMREE a pipeline in a product addressing (rare) inflammatory diseases

PRODUCT PIPELINE

PRODUCT	DRUG CLASS	INDICATION	STATUS	LAUNCH YEAR (EXPECTED)	PARTNER	GLOBAL PEAK SALES
NEUROMUSCULAR DISEASES						
AGAMREE (VAMOROLONE)	DISSOCIATIVE STEROID	DUCHENNE MUSCULAR DYSTROPHY (DMD)	APPROVED IN US & EU	2024	NORTH AMERICA: CATALYST GREATER CHINA: SPEROGENIX	CHF 750+ MN
AGAMREE (VAMOROLONE)	DISSOCIATIVE STEROID	BECKER MUSCULAR DYSTROPHY (BMD)	PHASE II POC	TBD	NORTH AMERICA: CATALYST GREATER CHINA: SPEROGENIX	TBD
AGAMREE (VAMOROLONE)	DISSOCIATIVE STEROID	RARE PEDIATRIC DISEASES - TBD	TBD	TBD	NORTH AMERICA: CATALYST GREATER CHINA: SPEROGENIX	TBD

ESTIMATES AS OF 4 JUNE 2024 SOURCE: VALUATIONLAB ESTIMATES

AGAMREE – First-in-class steroid replacement in DMD without typical side effects
AGAMREE is targeted to replace current glucocorticoid (steroid) treatment in DMD patients with early-to-mid-stage disease to preserve muscle strength, however, without the typical steroid safety issues irrespective of their underlying genetic mutation. In 2020, Santhera exercised its option to obtain the global rights to AGAMREE in DMD and all other indications, including Japan and South Korea, which paves the way for partnering large non-DMD indications such as inflammatory bowel disease (IBD), asthma, or chronic obstructive pulmonary disease (COPD), juvenile dermatomyositis, (pediatric) ulcerative colitis, and lung cancers, among others. In 2021, the pivotal phase IIb "VISION-DMD" trial of vamorolone in DMD was successfully concluded with positive 24-week and 48-week results reported across multiple endpoints with a compelling safety profile compared to standard corticosteroid therapy. Based on these positive trial results and in the space of three months, AGAMREE received full regulatory approval for treating DMD in the US, EU and the UK. In January 2024, AGAMREE was first launched in Germany, followed by Austria in February and the US in March, with the rollout in the UK expected in the summer and other large European countries in early 2025. We forecast peak sales of AGAMREE to amount to CHF 750+ mn (including partner sales) in DMD alone.

Rationale for AGAMREE in DMD/BMD
AGAMREE is a novel first-in-class dissociative steroidal anti-inflammatory compound and is a close analog to prednisone, a conventional glucocorticoid. However, without the typical steroid side effects such as growth stunting, bone density loss, and mood disturbances. AGAMREE binds to the glucocorticoid receptor (GR) and, like conventional steroids, activates the anti-inflammatory (NFkB) response, but unlike conventional steroids, it does not activate gene transcription pathways, which are responsible for side effects. The differential activation of GR-mediated pathways results in therapeutic (anti-inflammatory) activity but does not activate the pathway

associated with glucocorticoid-class side effects. The lipophilic properties of AGAMREE allow it to integrate into the cell membrane to provide stability in an environment where stability is crucial due to the lack of dystrophin. Moreover, AGAMREE is an antagonist for mineralocorticoid receptors (for instance, spironolactone, which is given routinely to DMD patients to reduce the rate and risk for cardiomyopathy) and hence may have cardioprotective properties as opposed to glucocorticoids, which are agonists, adding to its favorable safety profile.

In the following section, we will provide in-depth analysis and forecasts for Santhera's key driver:

- **AGAMREE in DMD** (see the following page)

Forecasts & Sensitivity Analysis

AGAMREE (Duchenne muscular dystrophy)

Product Analysis

DMD peak sales of CHF 750+ mn - rNPV of CHF 48 per share

We forecast global peak sales of CHF 750+ mn (including partner sales) for AGAMREE in DMD, with first launches in the EU, UK, and US starting in 2024 and in the Greater China Region in 2025 with a method of use patent protection until at least 2029, a granted US polymorph patent extending US protection until 2040, next to 7-year (US) and 10-year (EU) orphan drug market exclusivity and additional 2-year pediatric exclusivity providing 12 years protection in the EU extending protection until 2035. We conservatively assume an annual treatment cost per patient of USD 100,000 in North America. In the EU and ROW, we apply a ~45% discount to the US pricing at EUR 52,000 in the EU and USD 57,000 in ROW, while in China, we apply a sharp 80% discount, amounting to USD 20,000 per patient. In North America, we assume a peak penetration rate of 40%, conservative sales milestones of CHF 84 mn, and royalties from Catalyst starting at 7% and gradually increasing to 12%. Note that Catalyst pays Santhera's royalty obligations to Idorsia and ReveraGen (~5-7%). In Europe (Santhera territories), we assume a peak penetration rate of 35%, 10% COGS, M&S costs for the specialist sales force peaking at around CHF 25-30 mn, and tiered royalty payments to Idorsia and ReveraGen of 5%, increasing to 7%. We expect a 25% peak market penetration rate in the Greater China region, 15% royalties, and CHF 34 mn milestones from Sperogenix. In the ROW, we assume similar terms as for the Greater China region from new commercialization partners. Our NPV for AGAMREE in DMD amounts to CHF 851 mn or CHF 48 per share with a WACC of 10% (see page 22)

AGAMREE launch signals a new era for Santhera

Vamorolone (branded "AGAMREE") is a first-in-class dissociative steroid with the promise to become a foundational chronic therapy for all Duchenne muscular dystrophy (DMD) patients, irrespective of their genetic mutation, with the potential of combination therapy. AGAMREE is expected to swiftly replace standard-of-care (SoC) steroids such as prednisone or deflazacort (branded "Emflaza" by PTC Therapeutics in the US with 2023 sales of USD 255 mn) due to its superior safety and tolerability profile while maintaining similar efficacy in prolonging muscle function and mobility.

In the space of just three months, AGAMREE received full approval for treating DMD in the US (October 2023), the EU (December 2023), and the UK (January 2024). AGAMREE is the only DMD treatment to receive full regulatory approval (except for Emflaza in the US), while all others have only received conditional or accelerated approval with additional clinical trial obligations to demonstrate a positive benefit-risk to maintain their marketing authorization. In January 2024, the first launch of AGAMREE occurred in Germany, followed by Austria in February and the US in March, with the UK rollout expected this summer. The other major European countries should follow in early 2025 once pricing and reimbursement have been established in each country.

AGAMREE is off to a flying start. The reception in Germany and Austria has been very positive, with strong demand and proactive requests from patients and caretakers. Within only a few months of availability, AGAMREE has been prescribed to around 150 patients (~5% of total DMD patients) in Germany and Austria, with initial sales for Q1 2024 amounting to CHF 2.1 mn. In the US, Catalyst reported that the early AGAMREE launch indicators highlight a promising product uptake surpassing initial expectations. Q1 2024 AGAMREE net product revenues of USD 1.2 mn were reported for the first two weeks of commercial availability in the US.

Santhera guides for sales of more than EUR 150 mn (>CHF 147 mn) for AGAMREE in Europe, in DMD alone. We forecast global peak sales for AGAMREE in DMD to amount to CHF 750+ mn (including partner sales). Further upside to our forecasts could come from treatment expansion in Becker muscular dystrophy (BMD) and other rare neuromuscular disorders and partnering in large inflammatory diseases such as asthma, chronic obstructive pulmonary disease (COPD), or inflammatory bowel disease (IBD) once an oral tablet formulation has been developed.

Santhera acquired the rights to AGAMREE from Idorsia, who became a stakeholder

AGAMREE was developed by its originator, the privately held US-based ReveraGen Biopharma Inc. (ReveraGen), with participation in funding and design of trials by several international non-profit foundations, including the US National Institutes of Health (NIH), the US Department of Defense, and the European Commission's "Horizon 2020" program, underlining the importance of this compound for treating a broad proportion of DMD patients with limited treatment options. Santhera obtained the option to in-license the global rights (excluding Japan/South Korea) of AGAMREE in all indications, particularly DMD, from Idorsia in 2018, which in turn had acquired the option for the exclusive global rights from ReveraGen in 2016. Idorsia became an anchor shareholder in Santhera with a current stake of 10.3%. In September 2020, Santhera secured the global rights for vamorolone in all indications via a direct license from ReveraGen, including Japan/South Korea. Idorsia and ReveraGen are eligible for up to USD 130 mn in sales milestone payments and increasing tiered low single to double-digit percentage royalties on sales. (see Appendix, page 26).

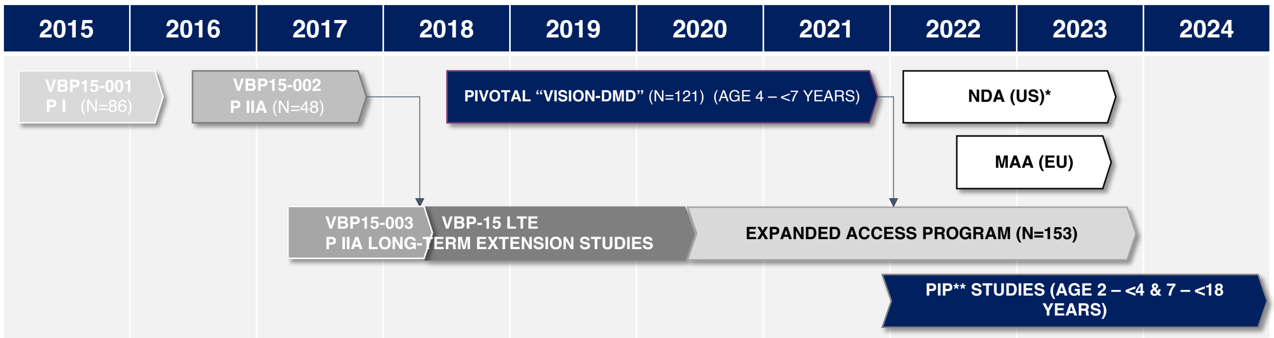
Developed to replace steroids and improve & extend efficacy with fewer side effects

AGAMREE was developed by ReveraGen as a first-in-class "dissociative" steroid – chemically separating the aspects of efficacy (clinical benefit) from safety concerns (side effects). AGAMREE is a close analog to prednisone, a conventional glucocorticoid (steroid), however, without the typical steroid side effects. AGAMREE is set to replace mainstay steroids such as prednisone or deflazacort (branded Emflaza in the US), the current foundational treatment in DMD patients with early- and mid-stage disease.

Early initiation of steroids preserves muscle function and strength, delaying time to loss of function by 2-3 years. Moreover, steroid treatment is associated with a reduction in all-cause mortality, new onset, and progressive cardiomyopathy (the main cause of death in DMD patients). DMD patients are diagnosed at age 3-5 years and typically remain on steroids up until the loss of ambulation (at around 11-12 years of age), with doses often down-titrated due to tolerability issues over time. Chronic use of steroids is often hampered by side effects such as weight gain, Cushingoid appearance (facial puffiness), severe mood and behavioral changes, excessive hair growth, growth stunting, decrease in bone density and spinal fractures, cataracts, and upper respiratory infection and cough. As a result, a significant portion of patients are not offered or start steroid treatment, start treatment late, are treated with sub-optimal doses due to side effects, or stop treatment prematurely.

Extensive development program resulted in full approval in the US, EU, and UK
AGAMREE (vamorolone) received full regulatory approval in the US, EU, and UK based on an extensive clinical development program, demonstrating robust efficacy and safety data required for approval. The program currently has 200 patient-years exposure in 160 DMD patients treated with AGAMREE for up to 7 years (see graph below).

COMPREHENSIVE CLINICAL DEVELOPMENT PROGRAM VAMOROLONE IN DMD



* ASSUMES PRIORITY REVIEW; ** PIP = PEDIATRIC INVESTIGATIONAL PLAN
SOURCE: SANTHERA PHARMACEUTICALS

All trials (except for the ongoing PIP Study with results due in 2024) concluded positively, with AGAMREE demonstrating similar efficacy as in-market steroids with a clear dose-response; however, with a superior safety and tolerability profile after 24 weeks, 48 weeks, and 30 months of treatment (see Appendix, page 28)

“VISION-DMD” shows clinically meaningful improvement in functional outcomes
In the pivotal VISION-DMD trial, the primary endpoint was reached. The time-to-stand (TTSTAND) velocity of the highest dose of AGAMREE (6 mg/kg/day) showed a clinically meaningful difference of 0.06 rises per second compared to placebo (prednisone 0.75 mg/kg/day) with high statistical significance (p=002) after 24 weeks of treatment. This is a 23% improvement in TTSTAND compared to placebo and is expected to delay the time to loss of ambulation by 2-3 years. Four out of five pre-specified secondary endpoints after 24 weeks of treatment showed statistical significance, including the TTSTAND velocity of the low dose (2 mg/kg/day), the 6-minute walk test (6MWT) of both the high and low dose, and time-to-run-walk (TTRW) velocity in the high dose (the low dose missed statistical significance with a p-value of 0.103). Other explanatory endpoints, including the time-to-climb (TTCLIMB) velocity and the North Star Ambulatory Assessment (NSAA), reached statistical significance for both the high and low doses after 24 weeks of treatment.

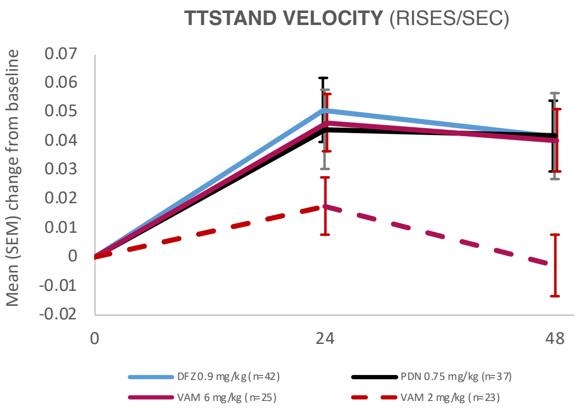
OVERVIEW EFFICACY DATA VAMOROLONE "VISION-DMD" TRIAL PERIOD 1 (WEEK-24, PRIMARY ANALYSIS)

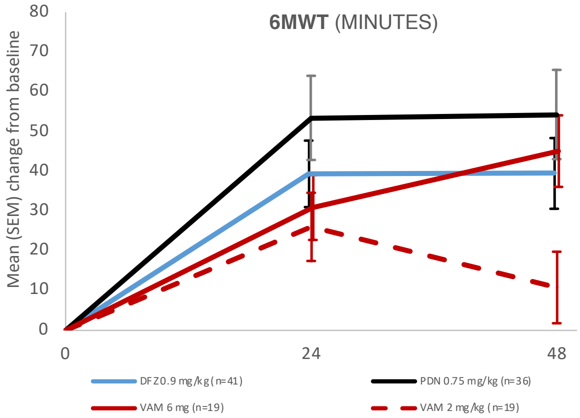
RANK	ENDPOINT	COMPARISON	DIFFERENCE VS. PLACEBO	P-VALUE
PRIMARY ENDPOINT:				
1	TTSTAND VELOCITY	VAMOROLONE HIGH DOSE VS. PLACEBO	0.06 RISES/SECOND	0.002
SECONDARY ENDPOINTS:				
2	TTSTAND VELOCITY	VAMOROLONE LOW DOSE VS. PLACEBO	0.04 RISES/SECOND	0.017
3	6MWT DISTANCE	VAMOROLONE HIGH DOSE VS. PLACEBO	42 METERS	0.003
4	6MWT DISTANCE	VAMOROLONE LOW DOSE VS. PLACEBO	37 METERS	0.009
5	TTRW 10 METERS VELOCITY	VAMOROLONE HIGH DOSE VS. PLACEBO	0.24 METER/SECOND	0.002
6	TTRW 10 METERS VELOCITY	VAMOROLONE LOW DOSE VS. PLACEBO	0.13 METER/SECOND	0.103 NS

TTSTAND = TIME TO STAND; 6MWT = 6 MINUTE WALK TEST; TTRW = TIME TO RUN/WALK; NS = NON-SIGNIFICANT; HIGH DOSE = 6 MG/KG/DAY; LOW DOSE = 2 MG/KG/DAY
SOURCE: VALUATIONLAB, SANTHERA PHARMACEUTICALS

There was comparable efficacy between vamorolone high dose versus prednisone. A durable treatment effect was maintained over 48 weeks treatment and no loss of efficacy was seen when switching from prednisone to vamorolone. An improved safety profile was already observed with vamorolone compared to prednisone in the first 24 weeks, with fewer and milder treatment-emergent adverse events (TEAEs) with high-dose vamorolone, including behavioral problems. Low-dose vamorolone had placebo-like TEAEs.

Independent “FOR-DMD” Study provides positive external comparator data
AGAMREE’s data package for DMD was complemented by the independent **FOR-DMD Study (n=196)** an external, academic-run, double-blind, randomized comparator trial with pre-specified efficacy and safety analysis comparing DMD patients on 1) AGAMREE (Study VBP-15LTE (n=46) 30 months, and VISION-DMD (n=121) Period 1 (24-week) and Period 2 (48-week), and the steroid comparator group (n=196 consisting of 2) prednisone 0.75 mg/kg/day, 3) prednisone 0.75 mg/kg/day 10 days on and 10 days off, and 4) deflazacort 0.90 mg/kg/day (branded Emflaza in the US).





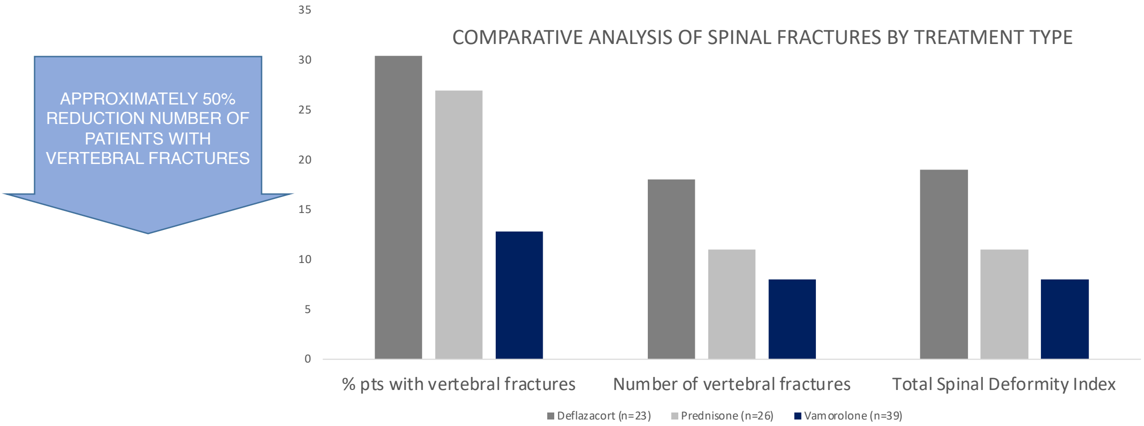
SOURCE: SANTHERA PHARMACEUTICALS

Propensity-matched cross-study comparisons between the VISION-DMD trial pre-specified analyses and the independent FOR-DMD Study external control show comparable efficacy for the AGAMREE high dose (6 mg/kg/day) in terms of TTSTAND and 6MWT compared to placebo.

In the cross-study comparison between Study VBP-LTE (long-term extension trial) and the FOR-DMD Study, fewer and less severe spinal fractures were observed with AGAMREE compared to classical steroids over 2 ½ years of treatment. Treatment with AGAMREE resulted in approximately 50% fewer spinal fractures.

FEWER AND LESS SEVERE SPINAL FRACTURES WITH VAMOROLONE OVER 2.5 YEARS

VAMOROLONE VS. "FOR-DMD", MATCHED COMPARISION, CENTRAL READING USING MODIFIED GENANT



SOURCE: SANTHERA PHARMACEUTICALS

Unlike classical steroids, AGAMREE does not negatively impact bone formation or bone remodeling biomarkers. A rapid recovery was observed after switching from prednisone.

AGAMREE clinical development program demonstrates a strong value proposition

As seen above, the extensive clinical development program demonstrated a strong value proposition for AGAMREE, including:

- **Durable efficacy compared to standard of compare steroids**
    - Statistically significant efficacy compared to placebo at 24 weeks for both low-dose (2mg/kg/day) and high-dose (6 mg/kg/dose).
    - No loss of efficacy when switching from prednisone to AGAMREE
    - Long-term efficacy of high-dose AGAMREE comparable to steroids at 48 weeks
- **Preserved bone health with AGAMREE, unlike with steroids**
    - Normal bone turnover biomarkers and a reduction of risk of spinal fractures with long-term treatment compared to steroids.
    - Normalized height trajectory comparable to placebo and no stunted growth as seen with steroids
- **Improved safety profile compared to prednisone evident in the first 24 weeks**
    - Placebo-like treatment-emergent adverse events (TEAEs) with low-dose AGAMREE.
    - Fewer and milder TEAEs with high-dose AGAMREE compared to prednisone, including behavioral problems.
- **Individualized dose adjustments minimize side effects and maintain LT therapy**
    - Effective 3-fold (2, 4, or 6 mg/kg/day) dose range with a dose-dependent safety profile allows for individualized dose adjustment to best manage tolerability and maintain long-term treatment.

Consequently, AGAMREE received full approval for treating DMD patients in the US (October 2023) for all patients of age 2 years and older and for all patients of age 4 years and older in the EU (December 2023) and in the UK (January 2024). The label allows patients to be switched from oral steroid treatment such as prednisone or deflazacort (branded Emflaza in the US) to AGAMREE without treatment interruption or a period of prior steroid dosage reduction to minimize the risk for adrenal insufficiency. Patients switching after long-term treatment with oral steroids should start at the high-dose (6 mg/kg/day) AGAMREE.

Emflaza provides a substantial USD 255 mn switch opportunity for AGAMREE

AGAMREE's superior safety and tolerability profile, which has efficacy similar to prednisone and deflazacort, provides a substantial switch opportunity globally. In particular, the US presents a large switch opportunity. Deflazacort was never approved in the US despite being widely generically available in the EU and large parts of the world until Marathon Pharmaceuticals developed it specifically for DMD and branded it “Emflaza.” In February 2017, Emflaza became the first steroid that the FDA approved to treat all forms of DMD in patients 5 years and older. Emflaza’s US launch was far from smooth. PTC Therapeutics bought the rights from Marathon in April 2017 amid controversy over its high pricing strategy. Since then, US sales for Emflaza have grown substantially to an estimated USD 255 mn (+17%) in 2023, despite having typical steroid side effects hampering early and long-term use. Going forward, Emflaza sales are expected to decline sharply with the entry of generic versions in the US. India’s Aurobindo received FDA approval for its generic version of Emflaza. Emflaza’s only remaining FDA exclusivity covers DMD patients ages 2 to 5 years, which expires in June 2026.

Potential to combine with in-market DMD drugs irrespective of genetic mutation

AGAMREE has been developed as a foundational chronic treatment for patients with DMD, irrespective of genetic mutation, with an excellent safety and tolerability profile. It can be given to DMD patients irrespective of the underlying genetic mutation with the potential of combination therapy, including Sarepta’s exon-skipping therapies, EXONDYS 51, VYONDYS 53 or AMONDYS 45, its gene therapy Elevidys, and NS Pharma’s exon-skipping therapy Viltepso. Note that all these therapies are only available in the US and have received accelerated approval from the FDA. Additional clinical trials are required to confirm the clinical benefit of these therapies and maintain the marketing authorization. PTC Therapeutics’ nonsense mutation DMD therapy Translarna, which received conditional approval in the EU in 2014, will be re-evaluated by the Committee for Medicinal Products for Human Use (CHMP) to remain on the market after the European Commission made the rare decision not to adopt the negative opinion of the CHMP on the renewal of Translarna’ conditional approval. Translarna EU sales in 2023 amounted to USD 179 mn.

Gene therapies emerging – Sarepta’s Elevidys leads the pack but has issues

Gene therapy is an area that has shown great promise. However, the last few years show that there remain challenges to delivering these potential therapies safely and effectively to all DMD patients. Gene therapy safety in DMD and beyond has come under more intense scrutiny after alarming side effects were seen in clinical trials. Sarepta, alongside Pfizer and Solid Biosciences, has been leading the development of gene therapies, but side effects and disappointing efficacy have slowed progress in several clinical programs. The frontrunner in gene replacement therapy for DMD is Sarepta’s Elevidys (delandistrogene moxeparvovec). In June 2023, it received accelerated FDA approval to treat ambulatory DMD patients aged 4 through 5 years with a confirmed mutation in the DMD gene based on evidence showing it led to the production of the protein micro-dystrophin, which the FDA judged was reasonably likely to translate to clinical benefits. Initial pricing was announced at USD 3.2 mn for a single, lifetime treatment. As a condition of approval, the FDA required a confirmatory trial to verify efficacy. In October 2023, the confirmatory phase III “EMBARK” trial failed its primary endpoint to show a statistically significant difference from placebo on the North Star Ambulatory Assessment functional scale. While typically a negative primary result would necessitate further clinical trials, Sarepta argues the collective data in a number of other (secondary) measures actually supports the use of Elevidys and filed for expansion of the treatment. The FDA has set a PDUFA date of 21 June 2024 to convert the accelerated approval to full approval and expand the labeling beyond current use in children aged 4 and 5 or withdraw the accelerated approval.

Gene therapies likely not a single-shot cure – steroids still play an important role
Despite the accelerated approval of Elevidys, it is not yet known how well the micro/mini-dystrophins will function as a replacement for standard dystrophin, how long the production of the micro/mini-dystrophin will last, and what the impact is on disease progression. In short, it is too early to conclude that gene therapies are single-shot cures. More frequent dosing seems plausible to maintain treatment. effect This has great implications for the pricing and reimbursement of gene therapy. We believe steroids will remain a foundational therapy in DMD as these can be given irrespective of mutation (frequently used in combination with exon skippers and readthrough therapies) and even now upon initiation of gene replacement therapies. We believe multi-drug therapy with steroids and other DMD therapies will remain, even when more gene therapies are approved, with AGAMREE well-positioned to be the steroid of choice due to its superior safety and tolerability profile compared to conventional steroids such as prednisone or deflazacort.

AGAMREE use could potentially be expanded to Becker muscular dystrophy (BMD)
Duchenne and Becker muscular dystrophy (BMD) are caused by mutations in the dystrophin protein. BMD is a progressive muscle-wasting disease like DMD but is typically milder. In DMD, functioning dystrophin is completely absent in muscle, while in BMD, there is some dystrophin present, although not enough for completely normal muscle function, leading to variable onset (from 5 to 60 years of age) and progression of muscle weakness. The incidence of BMD is estimated to range between 1 in 18,000 and 1 in 30,000 male births with the number of patients approximately one-third of DMD patients. The mechanisms of action provide the basis for AGAMREE efficacy, as demonstrated in the positive pivotal "VISION-DMD" trial in the more severe muscle-wasting disease DMD, which is felt to be highly relevant to BMD. In addition, AGAMREE is hypothesized to increase dystrophin protein levels in BMD via inhibition of microRNAs that deleteriously target dystrophin, and this may further complement the mechanism of action, specifically in BMD. There are currently no approved drugs for BMD with very few clinical development programs underway. Steroids are often not accepted by BMD patients due to their side effects. AGAMREE, with its more favorable side effect burden, may provide a valuable new treatment option.

A pilot trial of AGAMREE in BMD started with results due by the H1 2025
In September 2021, ReveraGen received a USD 1.2 mn grant from the FDA under their "Clinical Studies of Orphan Products Addressing Unmet Need of Rare Disease (R01)" grants program to start a clinical trial of AGAMREE in adults and children with Becker muscular dystrophy (BMD), further underlining the potential in other (rare) muscular disorders. The phase II pilot trial of AGAMREE in BMD has started enrolling patients at sites in Padova (Italy) and Pittsburgh (US), with results due by H1 2025. This is a double-blind, placebo-controlled trial, with participants randomized to 2:1 vamorolone or placebo, to evaluate the safety, tolerability, and exploratory clinical efficacy of daily AGAMREE on motor function and established biomarker outcomes over a treatment period of 24 weeks in 39 males aged 18 or above and below 65 years with BMD. Given the early development stage, we currently exclude BMD from our AGAMREE forecasts.

AGAMREE has huge promise outside DMD, targeting multi-billion-dollar markets
Additional formulations of AGAMREE are planned to allow differential dosing and pricing for indications outside DMD and neuromuscular disorders. Conventional steroids such as prednisone, deflazacort, or budesonide are widely prescribed to treat inflammatory diseases such as asthma, chronic obstructive pulmonary disease (COPD), ulcerative colitis, and inflammatory bowel disease (IBD), among others. These indications are too large for Santhera (and potentially Catalyst) to target alone and require substantial financial resources and know-how to develop these indications and sufficient marketing muscle to commercialize them successfully. Peak sales in these indications could be a multiple of AGAMREE in DMD and other rare neuromuscular diseases. However, the company needs to develop a tablet formulation before it can partner and develop AGAMREE in these large steroid indications.

Extensive protection through exclusivities and US polymorph patent up to 2040
The patent portfolio under exclusive license from ReveraGen includes granted use patents for AGAMREE in DMD until 2029, with a likely term extension of up to 5 years. It has also been granted Orphan Drug Designation in the EU (10-year market exclusivity) and the US (7-year market exclusivity) and has also received Rare Pediatric Disease designation by the FDA and Promising Innovative Medicine (PIM) status from the UK MHRA. Following its 2023 approval, AGAMREE will enjoy Orphan Drug exclusivity until 2030 in the US and 2035 in the EU, including a 2-year pediatric extension.

In August 2022, the US Patent and Trademark Organization (USPTO) granted a new patent to protect AGAMEE crystal form (polymorph) and suspension with expiry in July 2040. The AGAMREE molecular structure has been disclosed in the past, preventing the composition of matter protection. However, the molecule is known to be stable in just one crystal form, polymorphic form I. No other crystal form is stable and suitable. The granted US patent claims a crystalline form of AGAMREE (polymorphic form I) and an oral pharmaceutical suspension comprising AGAMREE polymorphic form I. The presence of polymorphic form I can be easily detected in infringement cases.

Catalyst acquired North American commercialization rights in a frontloaded deal
In June 2023, an exclusive license and collaboration agreement was announced with Catalyst Pharmaceuticals (Catalyst) [NASDAQ: CPRX] for the rights of AGAMREE, including indications beyond DMD for North America (US, Canada, Mexico) in a deal valued at up to USD 231 mn plus royalties. The deal was frontloaded with a relatively high USD 90 mn upfront payment (USD 75 mn in cash, USD 15 mn equity investment in Santhera resulting in an 11.2% stake) followed by an additional USD 10 mn cash milestone payment, and USD 26 mn to pay for approval-related regulatory milestones to Idorsia and ReveraGen upon the US approval on 26 October 2023. Santhera is entitled to potential sales milestone payments of up to USD 105 mn. Catalyst will pay Santhera up to low-teen percent royalties on sales and corresponding third-party royalty obligations (single-digit to low double-digit percentage royalties) on AGAMREE sales in North America (see Appendix, page 26).

The upfront proceeds allowed for the repayment of all short-term debt with Highbridge and the overall strengthening and de-risking of the balance sheet, thereby extending Santhera's cash runway into Q1 2025 (excluding the maturing convertible bonds in August 2024).

Catalyst has a track record in rare diseases - AGAMREE US launched in March 2024
Catalyst Pharmaceuticals is an experienced rare disease specialist with an excellent track record and the marketing muscle to successfully commercialize AGAMREE in DMD in North America with the willingness and sufficient funds to invest in other indications outside DMD. Santhera and Catalyst have established a joint steering committee (JSC) to undertake the joint clinical development of AGAMREE for global indications, in which both parties will participate in the development process and funding. The US launch of AGAMREE in DMD, which started at the end of March 2024, is supported by a comprehensive Patient Assistance Program to enhance patient enrollment and sales uptake. Catalyst reported that the early AGAMREE launch indicators highlight a promising product uptake surpassing initial expectations. In Q1 2024, net product revenues of USD 1.2 mn were reported for the first two weeks of commercial availability of AGAMREE.

US peak sales of more than CHF 450 mn in DMD alone
In the US, we forecast AGAMREE peak sales in DMD to reach CHF 456 mn, assuming a 40% peak market penetration and an annual treatment cost per patient of USD 100,000. We assume additional sales milestones of CHF 89 mn (eligible for up to USD 105 mn sales milestones) and tiered net sales royalties starting at 7% and gradually increasing to 12%. Catalyst pays for third-party royalty obligations (tiered single to low double-digit percentage royalties to Idorsia/ReveraGen).

The first launch of AGAMREE in Germany started the European rollout
On 15 January 2024, the first launch occurred in Germany, followed by Austria in February, the UK this summer, and the other European countries by early 2025. Santhera will commercialize AGAMREE through its own specialist sales force in the top-5 Europe (Germany, UK, France, Italy, Spain), the Benelux (Belgium, Netherlands, Luxemburg), and Switzerland and Austria and seek partners for the remaining EU countries. The company expects to increase its staff by up to 60 incremental employees over the next two years to expand its specialist sales force. For certain countries where Santhera chooses not to market AGAMREE directly, including certain European countries and Japan, it has granted Catalyst a right of first negotiation in partnering discussions.

Europe peak sales of CHF 195 mn (Santhera guides for > EUR 150 mn)
In the EU, we forecast AGAMREE peak sales in DMD to reach CHF 195 mn, assuming a 35% peak market penetration and an annual treatment cost per patient of EUR 52,000. Santhera guides for peak sales of >EUR 150 mn (>CHF 147 mn) in its territories. We assume COGS of 10%, peak M&S costs of around CHF 25-30 mn, and tiered single- to low double-digit percentage sales royalty payments to Idorsia and ReveraGen.

Greater China rights in a deal valued at USD 124 mn
Sperogenix acquired the AGAMREE rights for the Greater China Region (mainland China, Hong Kong, Macau, and Taiwan) in a deal valued at USD 124 mn plus double-digit royalties on net sales. The company expects approval in China in Q1 2025 thanks to receiving a Priority Review in China. The Greater China Region conservatively provides approximately CHF 100+ mn in peak sales based on an annual treatment cost per patient of USD 20,000 (an 80% discount to the US pricing) with a conservative 25% peak market penetration rate.

Forecasts & Sensitivity Analysis

AGAMREE (VAMOROLONE) - FINANCIAL FORECASTS FOR DUCHENNE MUSCULAR DYSTROPHY

INDICATION	PRESERVATION OF MUSCLE FUNCTION IN DMD WITH IMPROVED SAFETY AND TOLERABILITY PROFILE THAN EXISTING STEROIDS
DOSAGE	STARTING AT 6 MG/KG/DAY WITH THE POSSIBILITY TO REDUCE DOWN TO 2 MG/KG/DAY WHEN TOLERABILITY ISSUES OCCUR
PRICE	ANNUAL COST PER PATIENT: US: USD 100,000 (~10% PREMIUM TO EMFLAZA); EU: EUR 52,000 ; CHINA: USD 20,000; ROW: USD 57,000
STANDARD OF CARE	STEROIDS (OFF-LABEL) & EMFLAZA (US ONLY); EXONDYS 51/VYONDYS 53 ACCELERATED APPROVAL (US ONLY); TRANSLARNA CONDITIONAL APPROVAL (EU ONLY)
UNIQUE SELLING POINT	REPLACEMENT OF EXISTING STANDARD OF CARE STEROIDS, WHICH REDUCES THE DECLINE IN MUSCLE FUNCTION WITHOUT THE TYPICAL STEROID SIDE EFFECTS

7Ps ANALYSIS

PATENT	METHOD OF USE PATENT: 2028-2030 (EXTENSION UP TO 5 YEARS); ORPHAN DRUG EXCLUSIVITY: EU: 10-YEAR + 2-YEAR PEDIATRIC; US 7-YEAR, US POLYMORPH PATENT: 2040
PHASE	PHASE IIA COMPLETED (INCL. POSITIVE DATA LONG-TERM "VBP15-003" EXTENSION TRIAL); POSITIVE PIVOTAL PHASE IIB "VISION-DMD" RESULTS (24 & 48 WEEKS) REPORTED 2021
PATHWAY	ORPHAN DRUG DESIGNATION IN EU & US - US: LAUNCHED MAR 2024; EU: LAUNCHED GERMANY JAN 2024, EU ROLLOUT EARLY 2025; UK: LAUNCH SUMMER 2024
PATIENT	REDUCTION IN PROGRESSIVE DECLINE IN MUSCLE FUNCTION MEANS IMPROVED QUALITY OF LIFE FOR PATIENT WITH LESS COMPLICATIONS
PHYSICIAN	EFFECTIVE TREATMENT WITH GOOD TOLERABILITY THAT REDUCES THE DECLINE IN MUSCLE FUNCTION, WHICH LEADS TO LOSS OF MOBILITY AND PREMATURE DEATH
PAYER	SUBSTANTIAL SAVINGS DUE TO LESS HOSPITALIZATIONS, LESS COMPLICATIONS AND LOWER SUPPORTIVE CARE COSTS
PARTNER	GLOBAL RIGHTS ACQUIRED FROM IDORSIA/REVERAGEN; NORTH AMERICA: CATALYST AGREEMENT FOR USD 111 MN UPFRONTS, UP TO USD 105 MN MILESTONES, LOW-TEEN ROYALTIES

REVENUE MODEL

NORTH AMERICA (CATALYST PHARMA)	**2023E**	**2024E**	**2025E**	**2026E**	**2027E**	**2028E**	**2029E**	**2030E**	**2031E**	**2032E**	**2033E**
NUMBER OF DMD PATIENTS	17'233	17'577	17'929	18'287	18'653	19'026	19'407	19'795	20'191	20'595	21'007
GROWTH (%)	2%	2%	2%	2%	2%	2%	2%	2%	2%	2%	2%
DIAGNOSED DMD PATIENTS (95%)	16'371	16'698	17'032	17'373	17'721	18'075	18'436	18'805	19'181	19'565	19'956
PATIENTS TREATED WITH CORTICOSTEROIDS (70%)	11'460	11'689	11'923	12'161	12'404	12'652	12'906	13'164	13'427	13'695	13'969
PENETRATION RATE (%)	0%	3%	11%	21%	28%	33%	35%	37%	38%	39%	40%
TREATED DMD PATIENTS	0	351	1'311	2'554	3'473	4'175	4'517	4'871	5'102	5'341	5'518
ANNUAL COST OF THERAPY (USD)	100'000	100'000	100'000	100'000	100'000	100'000	100'000	100'000	100'000	100'000	100'000
ANNUAL COST OF THERAPY (CHF)	89'315	90'526	90'526	90'526	90'526	90'526	90'526	90'526	90'526	90'526	90'526
PATIENT COMPLIANCE (%)	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%
SALES (CHF MN) - BOOKED BY CATALYST PHARMA	**0**	**25**	**95**	**185**	**252**	**302**	**327**	**353**	**370**	**387**	**400**
CHANGE (%)			274%	95%	36%	20%	8%	8%	5%	5%	3%
ROYALTIES (%)	0%	7%	7%	9%	9%	11%	11%	12%	12%	12%	12%
ROYALTY INCOME (CHF MN)	0	2	7	17	23	33	36	42	44	46	48
UPFRONT AND MILESTONE INCOME (CHF MN)	98	7	0	14	0	27	0	0	36	0	0
MILESTONE PAYMENTS TO IDORSIA/REVERAGEN (CHF MN)	-23	0	0	0	0	0	0	0	0	0	0
M&S COSTS (CHF MN)	-8	0	0	0	0	0	0	0	0	0	0
PROFIT BEFORE TAX (CHF MN)	**70**	**9**	**7**	**30**	**23**	**60**	**36**	**42**	**81**	**46**	**48**
TAXES (CHF MN)	0	0	0	-2	-3	-12	-7	-8	-16	-9	-10
PROFIT (CHF MN)	**70**	**9**	**7**	**29**	**19**	**48**	**29**	**34**	**64**	**37**	**38**

EUROPE TOP 5, BENELUX, A, CH (SANTHERA SPECIALIST SALES FORCE)	**2023E**	**2024E**	**2025E**	**2026E**	**2027E**	**2028E**	**2029E**	**2030E**	**2031E**	**2032E**	**2033E**
NUMBER OF DMD PATIENTS	18'619	18'805	18'993	19'183	19'375	19'568	19'764	19'962	20'161	20'363	20'567
GROWTH (%)	1%	1%	1%	1%	1%	1%	1%	1%	1%	1%	1%
DIAGNOSED DMD PATIENTS (95%)	17'688	17'865	18'043	18'224	18'406	18'590	18'776	18'964	19'153	19'345	19'538
PATIENTS TREATED WITH CORTICOSTEROIDS (70%)	12'381	12'505	12'630	12'757	12'884	13'013	13'143	13'275	13'407	13'541	13'677
PENETRATION RATE (%)	0%	3%	9%	18%	25%	30%	32%	33%	34%	34%	35%
TREATED DMD PATIENTS	0	375	1'137	2'296	3'221	3'904	4'206	4'381	4'491	4'604	4'718
ANNUAL COST OF THERAPY (CHF)	50'453	50'551	50'551	50'551	50'551	50'551	50'551	50'551	50'551	50'551	50'551
PATIENT COMPLIANCE (%)	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%
SALES (CHF MN) - BOOKED BY SANTHERA	**0**	**15**	**46**	**93**	**130**	**158**	**170**	**177**	**182**	**186**	**191**
CHANGE (%)			203%	102%	40%	21%	8%	4%	3%	3%	2%
COGS (10%) (CHF MN)	0	-2	-5	-9	-13	-16	-17	-18	-18	-19	-19
TIERED ROYALTY PAYMENTS TO REVERAGEN (%)	5%	5%	5%	5%	5%	7%	7%	7%	7%	7%	7%
TIERED ROYALTY PAYMENTS TO REVERAGEN (CHF MN)	0	-1	-2	-5	-7	-10	-11	-12	-12	-12	-12
R&D COSTS (EXCL. IDORSIA/REVERAGEN PAYMENTS) (CHF MN)	-19	-20	-7	-5	-2	0	0	0	0	0	0
M&S COSTS (CHF MN)	-2	-11	-20	-24	-24	-24	-25	-25	-25	-25	-26
PROFIT BEFORE TAX (CHF MN)	**-20**	**-18**	**12**	**49**	**85**	**107**	**117**	**123**	**126**	**130**	**134**
TAXES (CHF MN)	0	0	0	-2	-13	-21	-23	-25	-25	-26	-27
PROFIT (CHF MN)	**-20**	**-18**	**12**	**47**	**72**	**86**	**94**	**98**	**101**	**104**	**107**

GREATER CHINA REGION (SPEROGENIX)	**2023E**	**2024E**	**2025E**	**2026E**	**2027E**	**2028E**	**2029E**	**2030E**	**2031E**	**2032E**	**2033E**
NUMBER OF DMD PATIENTS	58'901	59'490	60'084	60'685	61'292	61'905	62'524	63'149	63'781	64'419	65'063
DIAGNOSED DMD PATIENTS (70%)	41'230	41'643	42'059	42'480	42'905	43'334	43'767	44'205	44'647	45'093	45'544
PATIENTS TREATED WITH CORTICOSTEROIDS (60%)	24'738	24'986	25'235	25'488	25'743	26'000	26'260	26'523	26'788	27'056	27'326
PENETRATION RATE (%)	0%	0%	1%	4%	8%	12%	16%	20%	24%	26%	27%
TREATED DMD PATIENTS	0	0	252	943	2'059	3'120	4'202	5'305	6'429	7'035	7'378
ANNUAL COST OF THERAPY (CHF)	17'863	18'105	18'105	18'105	18'105	18'105	18'105	18'105	18'105	18'105	18'105
PATIENT COMPLIANCE (%)	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%
SALES (CHF MN) - BOOKED BY SPEROGENIX	**0**	**0**	**4**	**14**	**30**	**45**	**61**	**77**	**93**	**102**	**107**
CHANGE (%)				250%	114%	0%	52%	35%	26%	11%	6%
ROYALTY INCOME (15%) (CHF MN)	0	0	1	2	4	7	9	12	14	15	16
UPFRONT AND MILESTONE INCOME (CHF MN)	2	0	7	0	0	0	5	0	9	0	0
TIERED ROYALTY PAYMENTS TO REVERAGEN (CHF MN)	0	0	0	0	0	0	0	0	0	0	0
MANUFACTURING REVENUE (~17%) (CHF MN)	0	0	1	2	5	8	10	13	16	17	18
COGS (15%) (CHF MN)	0	0	-1	-2	-4	-7	-9	-12	-14	-15	-16
PROFIT BEFORE TAX (CHF MN)	**2**	**0**	**8**	**2**	**5**	**8**	**15**	**13**	**25**	**17**	**18**
TAXES (CHF MN)	0	0	0	0	-1	-2	-3	-3	-5	-3	-4
PROFIT (CHF MN)	**2**	**0**	**8**	**2**	**4**	**6**	**12**	**10**	**20**	**14**	**15**

REST OF WORLD (JAPAN & AUSTRALIA) (PARTNERS TBD)	**2023E**	**2024E**	**2025E**	**2026E**	**2027E**	**2028E**	**2029E**	**2030E**	**2031E**	**2032E**	**2033E**
NUMBER OF DMD PATIENTS	5'171	5'223	5'275	5'328	5'381	5'435	5'490	5'544	5'600	5'656	5'712
DIAGNOSED DMD PATIENTS (80%)	4'137	4'178	4'220	4'262	4'305	4'348	4'392	4'436	4'480	4'525	4'570
PATIENTS TREATED WITH CORTICOSTEROIDS (70%)	2'896	2'925	2'954	2'984	3'014	3'044	3'074	3'105	3'136	3'167	3'199
PENETRATION RATE (%)	0%	0%	0%	2%	4%	8%	15%	19%	22%	24%	25%
TREATED DMD PATIENTS	0	0	0	60	121	243	461	590	690	760	800
ANNUAL COST OF THERAPY (CHF)	50'909	51'600	51'600	51'600	51'600	51'600	51'600	51'600	51'600	51'600	51'600
PATIENT COMPLIANCE (%)	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%	80%
SALES (CHF MN) - BOOKED BY PARTNER(S)	**0**	**0**	**0**	**2**	**5**	**10**	**19**	**24**	**28**	**31**	**33**
CHANGE (%)				405%	89%	92%	36%	23%	14%	5%	-4%
ROYALTY INCOME (15%) (CHF MN)	0	0	0	0	1	2	3	4	4	5	5
UPFRONT AND MILESTONE INCOME (CHF MN)	0	0	0	9	0	0	0	2	3	0	0
TIERED ROYALTY PAYMENTS TO REVERAGEN (CHF MN)	0	0	0	0	0	0	0	0	0	0	0
PROFIT BEFORE TAX (CHF MN)	**0**	**0**	**0**	**9**	**1**	**2**	**3**	**5**	**7**	**5**	**5**
TAXES (CHF MN)	0	0	0	0	0	0	-1	-1	-1	-1	-1
PROFIT (CHF MN)	**0**	**0**	**0**	**9**	**1**	**1**	**2**	**4**	**6**	**4**	**4**

	2023E	**2024E**	**2025E**	**2026E**	**2027E**	**2028E**	**2029E**	**2030E**	**2031E**	**2032E**	**2033E**
GLOBAL PRODUCT SALES (CHF MN)	**0**	**41**	**145**	**294**	**417**	**515**	**577**	**631**	**673**	**706**	**730**
CHANGE (%)			256%	103%	42%	24%	12%	9%	7%	5%	3%
GLOBAL PROFIT (CHF MN)	**51**	**-9**	**26**	**87**	**96**	**142**	**137**	**147**	**191**	**159**	**164**
CHANGE (%)	-269%	-118%	-393%	230%	11%	47%	-3%	7%	30%	-17%	3%
WACC (%)	10%										
NPV TOTAL PROFIT (CHF MN)	**851**										
NUMBER OF SHARES (MN)	17.7										
RISK ADJUSTED NPV PER SHARE (CHF)	**48**										

SENSITIVITY ANALYSIS

		WACC (%)						
	CHF/SHARE	7	8	9	10	11	12	13
	970	75	70	65	61	57	53	50
	870	67	63	58	54	51	48	45
PEAK SALES (CHF MN)	770	60	55	52	**48**	45	42	39
	670	52	48	45	42	39	37	34
	570	44	41	38	36	33	31	29

ESTIMATES AS OF 4 JUNE 2024

SOURCE: VALUATIONLAB ESTIMATES

Unique Selling Point

The first-in-class dissociative steroid with the potential to replace DMD standard-of-care steroid therapy (prednisone, deflazacort) due to a superior safety and tolerability profile with similar efficacy. AGAMREE can be given to DMD patients irrespective of the underlying genetic mutation and potentially in combination with other DMD treatments, including potential gene therapies, such as Sarepta's recently approved Elevidys.

7P's Analysis

Patent: In the US, we expect extensive patent protection until 2040 based on a granted new US polymorph patent. The method of use patents expires in 2029 with a likely extension of up to 5 years. In the EU, we expect protection until 2035 based on 10-year orphan drug exclusivity and 2-year pediatric exclusivity from the approval date.

Phase: The single pivotal phase IIb "VISION-DMD" trial concluded in 2021 with positive 24-week (primary analysis) results reported in June and positive 48-week results reported in November. AGAMREE has been fully approved in the US, the EU, and the UK, and has been launched in Germany, Austria, the US and the UK rollout expected this summer and the other major European countries in early 2025 after pricing and reimbursement has been established in each country.

Pathway: AGAMREE enjoys orphan drug designation in both the EU and US, which is an important incentive to develop drugs for rare diseases by providing 10 years (EU) and 7 years (US) marketing exclusivity from the date of approval.

Patient: Slowing down of progressive muscle function leads to longer patient mobility. The superior safety and tolerability profile leads to better patient compliance with fewer complications for the patient, such as bone fractures, stunted growth, behavior changes, improved quality of life, and potentially prolonged life expectancy.

Physician: AGAMREE is targeted to replace standard-of-care steroid therapy (prednisone or deflazacort) with an effective steroid with a superior safety and tolerability profile, enhancing patient compliance and long-term outcomes. AGAMREE slows the progressive loss of muscle function and mobility and can be combined with other approved DMD drugs, such as exon skippers, readthrough therapies, and potentially new gene therapies.

Payer: A treatment that slows down progressive muscle function loss in DMD patients may save costs related to loss of mobility (e.g., wheelchair, special bed), rescue medication, supportive care, and hospitalization. Compared to mainstay steroids, it superior safety and tolerability profile should lead to fewer secondary complications such as bone fractures, increased patient compliance, and long-term treatment outcomes.

Partner: In North America, AGAMREE is being commercialized by Catalyst with up to USD 105 mn additional sales milestones and up to low-teen percentage royalties on sales. Santhera will sell AGAMREE through its own small specialist sales force in the top-5 Europe, the Benelux, Switzerland, and Austria. Sperogenix acquired the rights for the Greater China region with Santhera eligible for up to USD 104 mn additional milestones and double-digit royalties on sales. We assume Santhera will sign on commercialization partners for ROW at terms similar to the Sperogenix deal.

Income Statement

SANTHERA PHARMACEUTICALS — SHARE PRICE (CHF) 9.82

IFRS

INCOME STATEMENT (CHF MN)	2023	2024E	2025E	2026E	2027E	2028E	2029E	2030E	2031E	2032E	2033E
PRODUCT SALES (INCL. PARTNER SALES)	0	41	145	294	417	515	577	631	673	706	731
CHANGE (%)			256%	103%	42%	24%	12%	9%	7%	5%	4%
REPORTED SALES (SANTHERA TERRITORIES)	1	15	46	93	130	158	170	177	182	186	191
CHANGE (%)	-114%	1816%	203%	102%	40%	21%	8%	4%	3%	3%	2%
ROYALTIES (FROM PARTNER SALES)	3	2	7	19	28	42	48	58	63	66	69
CHANGE (%)	45%	-34%	305%	165%	46%	49%	15%	20%	9%	6%	4%
UPFRONT & MILESTONES	100	7	7	23	0	27	5	2	48	0	0
MANUFACTURING REVENUE	0	0	1	2	5	8	10	13	16	17	18
CHANGE (%)				274%	118%	52%	35%	26%	21%	9%	5%
OTHER REVENUES	0	0	0	0	0	0	0	0	0	0	0
REVENUES (EXCL. PARTNER SALES)	103	24	60	135	158	227	223	236	292	253	260
CHANGE (%)	1284%	-77%	150%	123%	18%	43%	-2%	6%	24%	-14%	3%
COGS (INCL. PAYMENTS TO IDORSIA)	-3	-2	-5	-11	-18	-23	-26	-29	-32	-34	-35
GROSS PROFIT	100	23	55	123	141	204	196	207	260	219	225
CHANGE (%)	2481%	-77%	144%	123%	14%	45%	-4%	5%	25%	-16%	3%
MARGIN (%)	96.9%	93.7%	91.5%	91.6%	88.9%	90.0%	88.3%	87.6%	89.0%	86.6%	86.5%
R&D	-19	-20	-19	-19	-18	-20	-20	-20	-21	-21	-21
CHANGE (%)	-39%	7%	-3%	1%	-8%	12%	1%	1%	1%	1%	1%
GENERAL & ADMINISTRATIVE	-21	-22	-23	-24	-24	-25	-25	-26	-26	-27	-27
CHANGE (%)	45%	5%	4%	3%	2%	2%	2%	2%	2%	2%	2%
MARKETING & SALES	-10	-11	-20	-24	-24	-24	-25	-25	-25	-25	-26
CHANGE (%)	-10%	12%	82%	20%	1%	1%	1%	1%	1%	1%	1%
OTHER OPERATING INCOME/(EXPENSE)	1	1	1	1	1	1	1	1	1	1	1
NET GAIN ON SALE OF DISPOSAL GROUP	18										
EBIT	69	-30	-6	57	75	135	127	137	189	146	151
CHANGE (%)	-232%	-143%	-78%	-979%	32%	81%	-6%	8%	38%	-22%	3%
MARGIN (%)	67%	-123%	-11%	42%	47%	60%	57%	58%	65%	58%	58%
EBITDA	72	-27	-3	60	78	139	130	140	192	150	155
CHANGE (%)	-271%	-137%	-88%	-1926%	31%	77%	-6%	8%	37%	-22%	3%
MARGIN (%)	70%	-110%	-5%	44%	49%	61%	59%	59%	66%	59%	60%
D&A	3	3	3	3	3	3	3	3	4	4	4
NET FINANCIAL INCOME/(EXPENSES)	-14	-6	-3	1	5	5	5	6	6	7	7
PROFIT/(LOSS) BEFORE TAXES	55	-35	-10	58	80	140	132	143	195	153	158
CHANGE (%)	-178%	-165%	-73%	-708%	37%	76%	-6%	8%	37%	-21%	4%
MARGIN (%)	53%	-147%	-16%	43%	50%	62%	59%	60%	67%	61%	61%
TAXES (EXCL. TAX LOSS CARRYFORWARDS)	0	0	0	-5	-17	-35	-34	-37	-48	-40	-41
TAX RATE (%)	0%	0%	0%	8%	21%	25%	26%	26%	25%	26%	26%
NET PROFIT/LOSS	55	-35	-10	53	63	105	98	106	147	113	117
CHANGE (%)	-177%	-165%	-73%	-660%	17%	68%	-7%	8%	39%	-23%	4%
MARGIN (%)	53%	-147%	-16%	40%	40%	46%	44%	45%	50%	45%	45%
EPS (CHF)	5.18	-2.81	-0.75	4.23	4.96	8.32	7.77	8.38	11.66	8.98	9.31

ESTIMATES AS OF 4 JUNE 2024 — SOURCE: VALUATIONLAB ESTIMATES

NOTE: On 31 December 2023, Santhera had a total of CHF 185.9 mn unrecorded tax loss carryforwards, which we anticipate the company will be able to use on future profits.

Ratios I Balance Sheet I Cash Flow Statement

SANTHERA PHARMACEUTICALS									SHARE PRICE (CHF)		9.82
IFRS											
RATIOS	**2023**	**2024E**	**2025E**	**2026E**	**2027E**	**2028E**	**2029E**	**2030E**	**2031E**	**2032E**	**2033E**
P/E		-3.5x	-13.0x	2.3x	2.0x	1.2x	1.3x	1.2x	0.8x	1.1x	1.1x
P/S		5.1x	2.1x	0.9x	0.8x	0.5x	0.6x	0.5x	0.4x	0.5x	0.5x
P/NAV		2.5x	3.1x	1.3x	0.8x	0.5x	0.3x	0.3x	0.2x	0.2x	0.1x
EV/EBITDA		-4.6x	-37.1x	2.0x	1.6x	0.9x	0.9x	0.9x	0.6x	0.8x	0.8x

PER SHARE DATA (CHF)	**2023**	**2024E**	**2025E**	**2026E**	**2027E**	**2028E**	**2029E**	**2030E**	**2031E**	**2032E**	**2033E**
EARNINGS	**5.18**	**-2.81**	**-0.75**	**4.23**	**4.96**	**8.32**	**7.77**	**8.38**	**11.66**	**8.98**	**9.31**
CHANGE (%)	-144%	-154%	-73%	-660%	17%	68%	-7%	8%	39%	-23%	4%
CASH	**2.87**	**1.85**	**1.35**	**6.20**	**12.76**	**24.15**	**34.91**	**46.49**	**62.21**	**74.62**	**87.47**
CHANGE (%)	1193%	-35%	-27%	359%	106%	89%	45%	33%	34%	20%	17%
DIVIDENDS	**0**	**0**	**0**	**0**	**0**	**0**	**0**	**0**	**0**	**0**	**0**
PAYOUT RATIO (%)	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%	0%
NET ASSET VALUE	**5.67**	**3.95**	**3.20**	**7.42**	**12.38**	**20.71**	**28.48**	**36.87**	**48.52**	**57.50**	**66.81**
CHANGE (%)	-179%	-30%	-19%	132%	67%	67%	38%	29%	32%	19%	16%

BALANCE SHEET (CHF MN)	**2023**	**2024E**	**2025E**	**2026E**	**2027E**	**2028E**	**2029E**	**2030E**	**2031E**	**2032E**	**2033E**
NET LIQUID FUNDS	**30**	**23**	**17**	**78**	**161**	**305**	**441**	**587**	**785**	**942**	**1'104**
TOTAL ASSETS	**110**	**103**	**96**	**157**	**240**	**384**	**520**	**666**	**864**	**1'021**	**1'183**
TOTAL SHAREHOLDERS' EQUITY	**60**	**50**	**40**	**94**	**156**	**261**	**359**	**465**	**612**	**726**	**843**
CHANGE (%)											
RETURN ON EQUITY (%)	91%	-71%	-24%	57%	40%	40%	27%	23%	24%	16%	14%
FINANCIAL DEBT	**28**	**0**	**0**	**0**	**0**	**0**	**0**	**0**	**0**	**0**	**0**
EMPLOYEES	56	76	98	113	114	115	116	118	119	120	121
- CHANGE IN %	*2%*	*35%*	*30%*	*15%*	*1%*	*1%*	*1%*	*1%*	*1%*	*1%*	*1%*

CASH FLOW STATEMENT (CHF MN)	**2023**	**2024E**	**2025E**	**2026E**	**2027E**	**2028E**	**2029E**	**2030E**	**2031E**	**2032E**	**2033E**
PROFIT / (LOSS) BEFORE TAXES	**55**	**-35**	**-10**	**58**	**80**	**140**	**132**	**143**	**195**	**153**	**158**
DEPRECIATION & AMORTIZATION	3	3	3	3	3	3	3	3	4	4	4
OTHER NON-CASH ITEMS	-11										
CASH FLOW FROM OPERATING ACTIVITIES	**47**	**-32**	**-6**	**61**	**83**	**144**	**136**	**146**	**198**	**157**	**162**
CASH FLOW FROM INVESTING ACTIVITIES	-18										
FREE CASH FLOW	**29**	**-32**	**-6**	**61**	**83**	**144**	**136**	**146**	**198**	**157**	**162**
CASH FROM FINANCING ACTIVITIES	0	25	0	0	0	0	0	0	0	0	0
EFFECTS OF EXCHANGE RATE CHANGES ON CASH	0										
CHANGE IN LIQUID FUNDS	**29**	**-7**	**-6**	**61**	**83**	**144**	**136**	**146**	**198**	**157**	**162**

ESTIMATES AS OF 4 JUNE 2024 SOURCE: VALUATIONLAB ESTIMATES

NOTE: Santhera guides for a cash reach into Q1 2025 (excluding maturing convertible bonds). Further capital of around CHF 50 mn may be needed to repay the outstanding convertible bonds (CHF 24.6 mn) due in August 2024 and to advance AGAMREE in new indications together with Catalyst. The company will prioritize debt financing and monetization of royalties over equity options, limiting potential share dilution at the current relatively low valuation. Santhera expects to reach breakeven on a cash basis by H1 2026.

APPENDIX

SANTHERA ADDITIONAL DATA

I) LICENSING AGREEMENTS:

AGAMREE (vamorolone):

1) **Idorsia (previously Actelion) - Global rights acquired in 2020**
   In September 2020, Santhera exercised its option to obtain the global rights to vamorolone (branded "AGAMREE") in DMD and all other indications, which it acquired from Actelion (now Idorsia) in 2018. Idorsia assigned its original exclusive agreement with ReveraGen to Santhera. The license gives Santhera global rights to AGAMREE, including Japan and South Korea.

   Remaining terms following the approval of AGAMREE in a nutshell:
   - **Sales milestones:** four one-time payments of up to USD 130 mn in aggregate
   - **Royalties on net sales:** tiered single-digit to low double-digit percentage of sales
   - **Regulatory milestones:** for three additional indications up to USD 205 mn in aggregate

2) **Catalyst - License and collaboration deal for North American rights in 2023**
   On 20 June 2023, an exclusive license and collaboration agreement was announced with Catalyst Pharmaceuticals (Catalyst) [NASDAQ: CPRX] for the rights of AGAMREE, including indications beyond DMD for North America (US, Canada, Mexico) in a deal valued at up to USD 231 mn plus royalties. The transaction was closed in July 2023. Catalyst is a commercial-stage US rare disease biopharmaceutical company. The deal was frontloaded with near-term cash payments of up to USD 126 mn with a relatively high USD 90 mn upfront payment from Catalyst ahead of the 26 October 2023 PDUFA date, indicating a strong conviction by Catalyst that AGAMREE would be approved for DMD.

   Terms of the Catalyst agreement for AGAMREE in North America:
   - Catalyst will commercialize AGAMREE in DMD with rights to all potential future indications in North America (US, Canada, Mexico).
   - Santhera and Catalyst will collaborate on joint development and funding of AGAMREE for additional indications beyond DMD.
   - Santhera received a USD 90 mn upfront payment at closing consisting of USD 75 mn in cash and a USD 15 mn equity investment (14,146,882 Santhera treasury shares resulting in an 11.2% stake) to support phase IV post-marketing trials in DMD and joint development of additional indications.
   - An additional USD 10 mn milestone payment plus USD 26 mn for approval-related regulatory milestones to third parties (Idorsia and ReveraGen) were paid by Catalyst upon US approval in 2023, with potential sales milestone payments of up to USD 105 mn remaining.
   - Catalyst will pay Santhera up to low-teen percent sales royalties and related third-party royalty obligations (single-digit to low double-digit percentage royalties to

Idorsia and ReveraGen) on AGAMREE sales in North America.

3) **Sperogenix - Exclusive rights for Greater China in 2022**
In January 2022, Santhera signed an exclusive licensing agreement with Sperogenix that acquired the rights for AGAMREE in DMD and other rare diseases for the Greater China Region (mainland China, Hong Kong, Macau, and Taiwan) in a deal valued at USD 124 mn. Santhera received a double-digit USD mn upfront cash payment plus short-term US regulatory milestones amounting to USD 20 mn combined and is eligible for up to USD 104 mn additional milestones and double-digit royalties on net sales.

OTHER PRODUCTS:

4) **Gene therapy - Agreement for LAMA2 MD signed with SEAL Therapeutics in 2022**
In February 2022, Santhera signed an agreement with SEAL Therapeutics, a spin-off company from the Biozentrum of the University of Basel, which will further develop a gene therapy approach intended for the treatment of laminin- alpha 2 (LAMA2)-deficient congenital muscular dystrophies ("LAMA2 MD", also known as "MDC1A"), a form of inherited and progressive muscle weakness with symptoms starting in newborns or infants. Santhera will be eligible for payments based on future proceeds of SEAL. Previous license agreements and preclinical collaborations that existed between Santhera and University Basel (initiated in May 2019), as well as Rutgers, The State University of New Jersey (both initiated in May 2020), have been terminated. SEAL will advance the gene therapy technology, building on existing research progress from the Biozentrum Basel and Rutgers, both previously partially funded by Innosuisse and/or Santhera. SEAL aims to attract a qualified pharmaceutical partner with experience in gene therapy technologies for clinical development and registration with the ultimate goal to make this innovative treatment approach available to patients with LAMA2 MD and their families.

5) **Raxone (idebenone) - Full divestment to Chiesi Group in 2023**
In July 2023, Chiesi Group acquired all assets and certain liabilities related to idebenone (branded "Raxone") in all indications worldwide from Santhera. This included Raxone in Leber hereditary optic neuropathy (LHON), for which Chiesi has already held exclusive license rights globally since 2019, except for North America and France. Under the terms of the agreement, Chiesi Group assumed the responsibility for the settlement agreed between Santhera and the French reimbursement authorities relating to Raxone in LHON amounting to EUR 25.3 mn. Santhera is eligible to participate in a potential marketing approval of Raxone in LHON in the US through variable payments in the single-digit percentage range on net sales or milestone payments of up to USD 10 mn. If Chiesi develops Raxone in non-ophthalmological indications, Santhera would be eligible for an additional milestone payment of USD 10 mn related to the approval in the US for the first non-ophthalmological indication and variable payments in the high single-digit percentage range on net sales if Chiesi develop

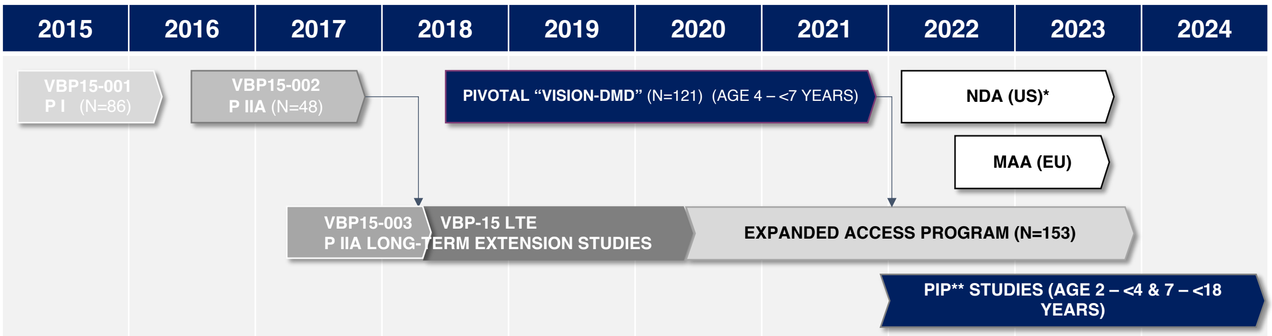
II) AGAMREE CLINICAL DATA:

Extensive development program in DMD resulted in approval in the US, EU, and UK
The companies designed an elaborate clinical development program for vamorolone (branded AGAMREE) for treating patients with DMD comprising of several clinical trials to demonstrate robust safety and efficacy data required for approval.

The extensive vamorolone DMD clinical development program currently has 200 patient-years exposure in 160 DMD patients treated with vamorolone for up to 6 years and consists of the following trials:

- **Study VBP15-001 (n-86):** a phase I trial to demonstrate initial safety and tolerability
- **Study VBP15-002 (n=48):** a 2-week open-label, 4-dose POC trial in steroid-naïve DMD patients aged 4-7 years, followed by **Study VBP15-003**, a 6-month, open-label, multiple-dose extension POC trial where all 48 patients from Study VBP15-002 were enrolled
- **Study VBP15-LTE (n=153):** a long-term, open-label extension trial up to 24 months where 46 patients who completed Study VBP15-003 were enrolled: positive results
- **"VISION-DMD" (n=121):** developed under FDA and EMA guidance as a pivotal, double-blinded, placebo-controlled phase IIb dose-ranging trial, consisting of a 24-week treatment period (Period 1), relevant for the primary analysis for both agencies and a 48-week treatment period (Period 2) required by the EMA to assess maintenance of treatment effect and additional longer-term safety data
- **PIP Study:** a pediatric investigational plan (PIP) to assess the safety and efficacy of vamorolone in younger (age 2 to under 4 years) and older (7 to above 18 years) DMD patients; ongoing, results due 2024

COMPREHENSIVE CLINICAL DEVELOPMENT PROGRAM VAMOROLONE IN DMD



* ASSUMES PRIORITY REVIEW; ** PIP = PEDIATRIC INVESTIGATIONAL PLAN
SOURCE: SANTHERA PHARMACEUTICALS

All trials (except for the ongoing PIP Study) concluded positively, with vamorolone demonstrating similar efficacy as in-market steroids with a clear dose-response, however, with a superior safety and tolerability profile after 6 months, 48 weeks, and 30 months of treatment.

Vamorolone's data package for DMD is complemented by the independent **FOR-DMD Study (n=196)** an external, academic-run, double-blind, randomized comparator trial with pre-specified efficacy and safety analysis comparing DMD patients on: 1) vamorolone (Study VBP-15LTE (n=46) 30 months, and VISION-DMD (n=121) Period 1 (24-week) and Period 2 (48-week), and the steroid comparator group (n=196 consisting of 2) prednisone 0.75 mg/kg/day, 3) prednisone 0.75 mg/kg/day 10 days on and 10 days off, and 4) deflazacort 0.90 mg/kg/day (branded Emflaza by PTC Therapeutics in the US).

Phase I program largely funded by DMD patient group organizations
In the US, pre-IND (investigational new drug) meetings were held with the FDA in October 2013, and the IND was filed in December 2014. Phase I clinical trials were completed in late 2015 in adult volunteers with SAD (single ascending dose) and MAD (multiple ascending dose) trials, which have been funded through venture philanthropy contracts by the Muscular Dystrophy Association (US), Joining Jack (UK), Duchenne Research Fund (UK) and Duchenne Children's Trust (UK). The DMD clinical program is being developed and run by a collaboration between the CINRG (Cooperative International Neuromuscular Research Group) and Newcastle University.

Phase IIa POC program provides encouraging data and the basis for the pivotal trial
The phase IIa POC development program consisted of two trials: **1) Study VBP15-002:** a 2-week open label, 4-dose trial in 48 steroid-naïve (not have taken prednisone or deflazacort) DMD patients aged 4 to 7 years; and **2) Study VBP15-003:** a 6-months, open label, multiple dose extension trial where all 48 patients from Study '002 were enrolled, with 46 completed.

1) Study VBP-15-002 showed that vamorolone was safe and well tolerated up to 6 mg/kg/day (approximately ten times the conventional steroid dose), with improved safety compared to steroids by reduction of insulin resistance, beneficial changes in bone turnover, and reduction in adrenal suppression.

2) Study VBP15-003 extension trial used timed function tests in comparison to conventional steroids and natural history data. Vamorolone showed dose-dependent efficacy in timed function tests comparable to conventional steroids with improved safety signals in biomarkers of insulin resistance, bone formation, and adrenal suppression.

The VBP15-003 phase IIa POC extension trial was a 6 months extension to the preceding VBP-002 phase IIa POC trial assessing the efficacy and safety of oral vamorolone in 48 patients with DMD aged 4 to <7 years. Vamorolone at all doses (0.25, 0.75, 2 and 6 mg/kg/day in 12 patients per treatment group) was safe and well tolerated. Mean changes from baseline to weeks 12 and 24 for the primary efficacy outcome, TTSTAND (time to stand) from supine measured as velocity, showed dose- and time-related improvements. The mean difference in change from baseline to week 24 was significant for the comparison of the 2 and 6 mg/kg/day groups to the lowest 0.25 mg/kg/day group (p=0.02 and p=0.04, respectively). The mean difference in change from baseline to week 24 was also significant for comparison of the 2 mg/kg/day vamorolone group to an untreated comparator cohort (p=0.04).

Motor function outcomes for secondary endpoints time to run/walk 10 meters (TTRW), time to climb stairs (TTCLIMB), 6-minute walk test (6MWT) and North Star Ambulatory Test showed a consistent pattern of vamorolone dose proportional improvement. Specifically, the 6 mg/kg/day dose showed significant improvements in TTRW (p=0.006) and 6MWT (p=0.002) compared to the lowest 0.25 mg/kg/day dose group. Vamorolone treatment led to increased serum levels of osteocalcin, a biomarker of bone formation, suggesting possible reduction of bone morbidities typically associated with corticosteroids. Biomarker outcomes for adrenal suppression and insulin resistance also indicated better tolerability of vamorolone treatment, relative to published studies of steroid therapy.

Extension Study VBP15-LTE shows long-term treatment effect and superior safety
46 patients who completed the Study VBP15-003 trial enrolled in **Study VBP15-LTE,** a long-term, open label extension trial up to 24 months of vamorolone treatment, with all doses increased to 2, 4 or 6 mg/kg/day. The Study VBP15-LTE completed in June 2020 with most boys opting for the high dose with no serious side effects attributable to vamorolone. In September 2020, Reveragen published encouraging detailed 18-month vamorolone treatment data, which was announced earlier at the World Muscle Society meeting in 2019. The trial demonstrated significant continued clinical improvement of all outcomes measured over the 18-month treatment period, similar to an external control group with corticosteroid-treated patients. Importantly, vamorolone did not show stunting of growth seen with deflazacort or prednisone with fewer steroid side effects such as mood disturbance, excessive hair growth and Cushingoid appearance (e.g., moon face), which we believe bodes well for the pivotal "VISION-DMD" trial.

Favorable 18-months Study VBP15-LTE data published in PLOS Medicine
In September 2020, the renowned PLOS Medicine journal published peer-reviewed and detailed open label data of the long-term **Study VBP15-LTE** of vamorolone in DMD patients treated for 18 months, demonstrating similar efficacy as conventional steroid treatment, however, without the hampering steroid-specific side effects.

Motor function and tolerability data from 23 DMD patients treated with oral vamorolone 2 or 6 mg/kg/day for at least 18 months in the ongoing VBP15-LTE trial were presented. The VBP15-LTE trial is an extension trial of the VBP15-003 phase IIa proof-of-concept (POC) trial in which 48 DMD patients were treated for 6 months over a broad dose range, from 0.25 up to 6 mg/kg/day, showing dose-related improvements in multiple gross motor outcomes. 46 patients who participated in the VBP-003 trial moved over to the 2 years open label long-term extension VBP15-LTE trial with all doses increased to 2 or 6 mg/kg/day of vamorolone.
Vamorolone treatment consistently and significantly improved motor function as measured by time to stand from supine (TTSTAND, $p=0.012$), time to run/walk 10 meters velocity (TTRW, $p<0.001$) and time to climb 4-stairs (TTCLIMB, $p=0.001$) from baseline to month 18. Motor function for vamorolone patients was also consistently better than outcomes for steroid naïve patients ($n=19$) from an external natural history trial with TTSTAND, $p=0.088$, TTRW, $p=0.003$, and TTCLIMB, $p=0.027$, all in favor of vamorolone-treated patients. When vamorolone-treated DMD patients were compared to age-matched prednisone-treated DMD patients from an external control group, vamorolone showed similar efficacy results as conventional steroids where both groups showed similar improvements in the gross motor outcomes noted above.

Importantly, vamorolone-treated DMD patients showed normal growth rates, less physician-reported weight gain and Cushingoid features (e.g. moon face) compared to published trials of prednisone or deflazacort. This is a key differentiator for vamorolone, where conventional steroids are widely prescribed to DMD patients in the early stage of disease and therefore at an early age, despite the steroid-specific side effects.

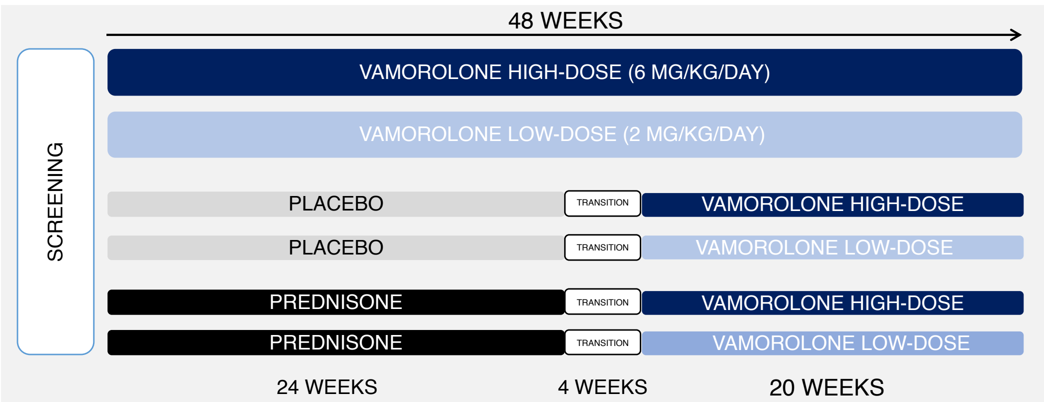
VBP15-LTE completed in 2020 – Most boys opt for high dose after 2 years
In June 2020, ReveraGen announced that it completed the long-term open label VBP15-LTE trial with 24 months treatment with vamorolone in patients with DMD. ReveraGen is still analyzing the efficacy data and plans to report the detailed results

in upcoming scientific conferences and publications. Including 6 months treatment in the preceding VBP15-003 trial, the company now has safety and efficacy data with vamorolone over a period of 2 ½ years in 41 boys with DMD with 106 patient-years of vamorolone exposure with no serious side effects attributable to vamorolone to date. All 46 patients who completed the VBP15-003 trial requested to continue vamorolone treatment in the long-term extension, rather than transition to conventional corticosteroids. The VBP15-LTE trial enabled dose escalation and de-escalation at the preference of the physician and family (suggested range 2.0 to 6.0 mg/kg/day). Of the 41 participants completing end-of-study visit after 24 months, 27 ended at 6.0 mg/kg/day (66%), 11 at 2.0 mg/kg/day (27%), and 3 at 4.0 mg/kg/day (7%). Thus, the majority of physicians/families chose treatment at the highest tested dose of vamorolone by the end of the VBP15-LTE trial. Positively, the large majority of the boys completing the 2-year VBP15-LTE trial have transitioned to Expanded Access Programs (USA, Canada, Israel) or compassionate use programs (UK, Sweden, Australia) to receive continued vamorolone treatment.

Positive long-term vamorolone treatment up to 2 ½ years reported
In April 2021, promising new 2.5-year treatment data was reported with vamorolone in DMD. These phase IIa long-term treatment data demonstrate a maintenance of treatment effect, equivalent to a delay of approximately two years in decline for TTSTAND (time to stand) velocity and confirm safety and tolerability benefits over the 2.5-year follow up period with significantly fewer corticosteroid-associated side effects than reported in other clinical trials with other steroids. Importantly, there were no reports of stunted growth, commonly reported in connection with corticosteroid treatment, over the entire follow up period.

Phase IIb "VISION-DMD" trial considered a pivotal trial for US and EU approval
The phase IIb trial "VISION-DMD" (VBP15-004) was developed under FDA and EMA guidance and is considered a pivotal trial for both US and EU approval. The 24-week placebo-controlled treatment period (Period 1) is relevant for the primary analysis for both agencies, with only the EMA in addition requesting 48-week data (Period 2) to assess

TRIAL DESIGN PIVOTAL "VISION-DMD" TRIAL VAMOROLONE IN DMD



SOURCE: SANTHERA PHARMACEUTICALS

maintenance of treatment effect and additional longer term safety data.

"VISION-DMD" was a phase IIb randomized, double-blind, parallel group, placebo- and active-controlled trial with a double-blind extension. The trial was conducted in 30 sites in the US, EU, Canada, Australia, and Israel and enrolled 121 ambulant boys from 4 to <7 years of age, who have not taken steroids (prednisone or deflazacort) who were randomized in 4 treatment groups: 1) low dose vamorolone (2 mg/kg/day), 2) high dose vamorolone (6 mg/kg/day), 3) mainstay prednisone 0.75 mg/kg/day, or 4) placebo. Treatment consisted of Period 1, a 24-week treatment period (weeks 1-24), a 4-week transition period (weeks 25-28) where the prednisone and placebo groups crossed-over to either low or high dose vamorolone, and Period 2, a second 20-week treatment period (weeks 28-48) and a 4-week dose-tapering period (weeks 49-52); with one visit per month.

The primary endpoint is efficacy measured by time to stand test (TTSTAND) of vamorolone at high dose 6.0 mg/kg/day vs. placebo group in change from baseline to week 24 assessment in Period 1. Secondary endpoints include safety, cardiac function, and additional efficacy measures (e.g., 6-minute walk test (6MWT), time to run/walk test (TTRW)).

The EMA's CHMP (Committee for Medicinal Products for Human Use) does not require a formal comparison between vamorolone and prednisone, but rather requests a global assessment of comparative efficacy and safety between vamorolone and prednisone without formal statistical hypothesis testing. First patient enrolment started in August 2018; positive 24-week (Period 1) results were reported in June 2021 followed by positive 48-week (Period 2) results reported in November 2021 (for details see below).

The pivotal phase IIb "VISION-DMD" trial successfully concluded with positive results after 24-week treatment (primary analysis) and extended 48-week treatment

PERIOD 1 (primary analysis after 24-week treatment):

Primary & Secondary endpoints reached over a broad dose range with good safety
On 1 June 2021, Santhera and partner ReveraGen reported positive 24-week (Period 1) topline results of the first treatment period of the pivotal phase IIb "VISION-DMD" trial where vamorolone demonstrated efficacy across primary and secondary endpoints and over a broad dose range, including the high dose (6 mg/kg/day) and low dose (2 mg/kg/day) as can be seen in the table below.

OVERVIEW EFFICACY DATA VAMOROLONE "VISION-DMD" TRIAL PERIOD 1 (WEEK-24, PRIMARY ANALYSIS)

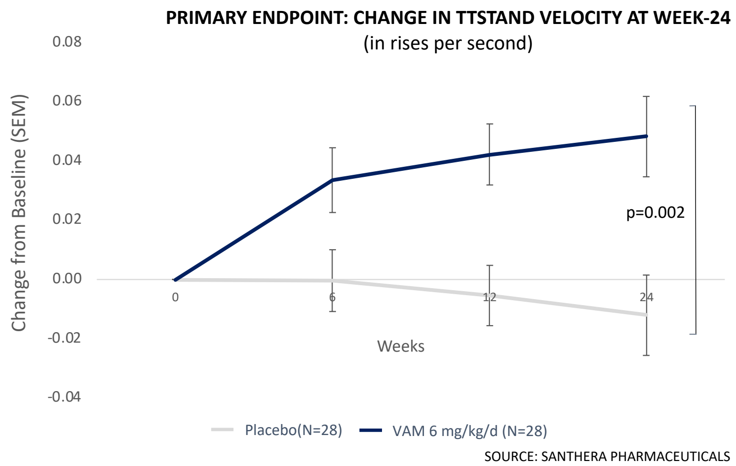
RANK	ENDPOINT	COMPARISON	DIFFERENCE VS. PLACEBO	P-VALUE
PRIMARY ENDPOINT:				
1	TTSTAND VELOCITY	VAMOROLONE HIGH DOSE VS. PLACEBO	0.06 RISES/SECOND	0.002
SECONDARY ENDPOINTS:				
2	TTSTAND VELOCITY	VAMOROLONE LOW DOSE VS. PLACEBO	0.04 RISES/SECOND	0.017
3	6MWT DISTANCE	VAMOROLONE HIGH DOSE VS. PLACEBO	42 METERS	0.003
4	6MWT DISTANCE	VAMOROLONE LOW DOSE VS. PLACEBO	37 METERS	0.009
5	TTRW 10 METERS VELOCITY	VAMOROLONE HIGH DOSE VS. PLACEBO	0.24 METER/SECOND	0.002
6	TTRW 10 METERS VELOCITY	VAMOROLONE LOW DOSE VS. PLACEBO	0.13 METER/SECOND	0.103 NS

TTSTAND = TIME TO STAND; 6MWT = 6 MINUTE WALK TEST; TTRW = TIME TO RUN/WALK; NS = NON-SIGNIFICANT; HIGH DOSE = 6 MG/KG/DAY; LOW DOSE = 2 MG/KG/DAY

SOURCE: VALUATIONLAB, SANTHERA PHARMACEUTICALS

The primary endpoint in change to TTSTAND shows clinically relevant improvement
The trial met its primary endpoint of superiority in change of TTSTAND (time to stand from supine positioning to standing) velocity with the high dose (6 mg/kg/day) of vamorolone versus placebo (p=0.002) with a treatment difference of 0.06 [95% CI: 0.02–0.10] rises/second from baseline (see graph below). This corresponds to a clinically relevant improvement in TTSTAND velocity in the vamorolone high dose group from 6.0 to 4.6

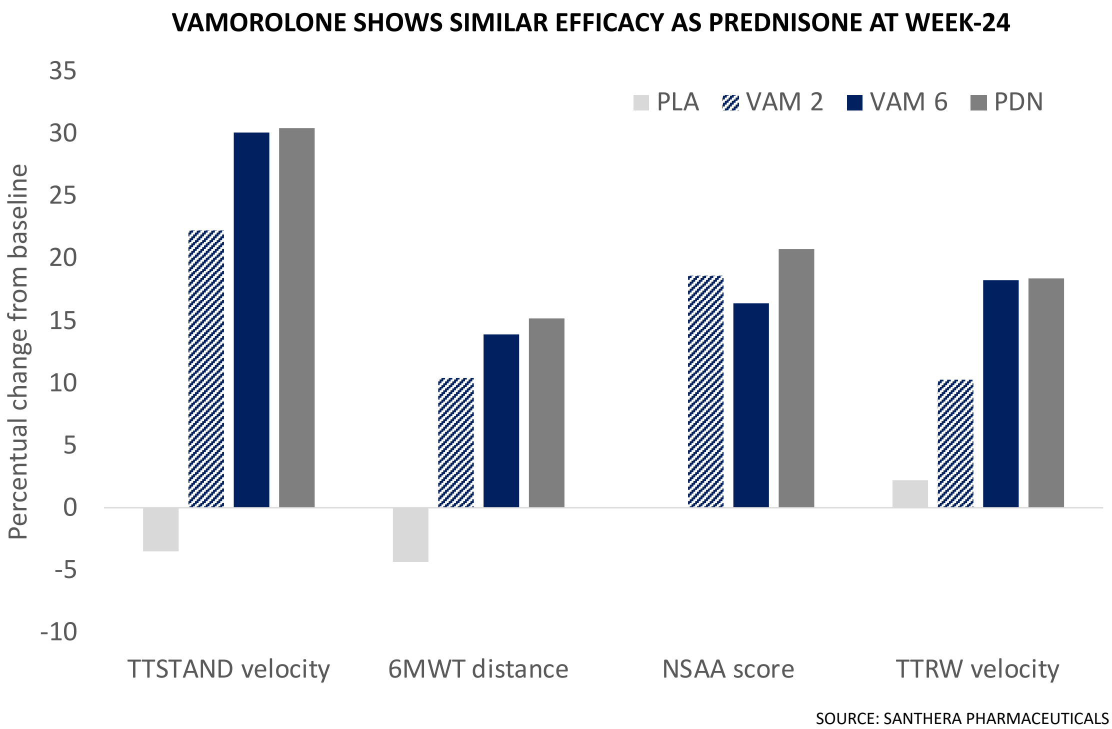
seconds (-23% change) and a corresponding deterioration in the placebo group from 5.4 to 5.5 seconds (+2% change. A treatment difference of 0.05 rises per second is predictive of a delay of 2 to 3 years in time to loss of ambulation.



RISE TIME (sec)	BL	W 24	% CHANGE
VAM 6 mg/kg/d	6.0	4.6	- 23%
PLACEBO	5.4	5.5	+ 2%

SOURCE: SANTHERA PHARMACEUTICALS

Consequently, the clinical results establish the efficacy of vamorolone over a wide, threefold dose range from 2 to 6 mg/kg/day. No statistically significant differences were observed between high dose vamorolone and mainstay steroid prednisone across the various endpoints (see graph below).



Favorable safety and tolerability profile compared to prednisone…

The trial completion rate in Period 1 at 24 weeks was 94% (or 114 of 121 participants). Vamorolone at both low and high doses showed a favorable safety and tolerability profile. In the vamorolone groups, no grade 3 or higher TEAEs (treatment emergent adverse events) or adverse events leading to trial discontinuation were observed. The total number of TEAEs was lower for low dose (events n=96) and high dose vamorolone (n=91) groups compared to prednisone (n=120). In a prespecified analysis of clinically relevant adverse events (moderate, severe, serious or leading to discontinuation due to safety), defined for the EMA and conducted by Santhera, high dose vamorolone was significantly superior to prednisone (n=6 vs n=19, p=0.02).

…without the feared stunted growth seen in chronic steroid use
Stunting of growth is a major concern of families and patients treated with corticosteroids and a major reason for not starting or for discontinuing efficacious steroid treatment. As previously published, in open-label trials of 2.5 years duration (113 patient years), vamorolone did not show stunting of growth as reported with conventional corticosteroids. This was validated in the pivotal "VISION-DMD" trial in Period 1 where high dose vamorolone compared to prednisone (0.75 mg/kg/day) showed a statistically significant difference in growth velocity (p=0.02). The strength of evidence for both efficacy and safety of vamorolone over such a wide dose range from 2 to 6 mg/kg/day allows clinicians to individually tailor treatment of Duchenne patients by starting at the high dose of vamorolone with equivalent efficacy to daily prednisone and titrate the dose according to how well the treatment is tolerated whilst maintaining optimal efficacy. This approach may allow patients to avoid side effects that currently lead to discontinuing steroid treatment and staying on treatment for longer.

PERIOD 2 (maintenance of treatment effect and safety after 48-weeks treatment):

Vamorolone efficacy established in Period 1 maintained in Period 2 with good safety
In Period 2, 114 subjects continued for another 24 weeks, where those on vamorolone low dose (2 mg/kg/day) and high dose (6 mg/kg/day) continued to end of the trial on these doses, and those on prednisone and placebo had been previously randomized to receive vamorolone low or high dose after a 4-week tapering period. 112 or 98% of patients completed the trial. Efficacy assessments at Period 2 (week 48) included Time to Stand (TTSTAND) velocity, 6-Minute Walk Test (6MWT), Time to Run/Walk 10 meters (TTRW) velocity, and North Star Ambulatory Assessment (NSAA).

Vamorolone during the trial showed sustained efficacy across multiple endpoints
Vamorolone high dose showed maintenance of efficacy across all parameters until trial end at week 48 and was statistically superior to low dose for TTSTAND velocity and 6MWT but not for TTRW velocity or NSAA. For patients who continued the same dose of vamorolone throughout the trial, the safety profile was consistent between periods with no increase in frequency or severity of adverse events being observed over time.

Vamorolone efficacy established in Period 1 was maintained in Period 2
The size of effect (change from baseline) observed at the primary endpoint in Period 1 at week 24 for vamorolone high dose was maintained in Period 2 at week-48 for TTSTAND velocity (0.052 vs 0.045 rises/s), NSAA (3.4 vs 3.5 points), TTRW velocity (0.29 vs 0.23 m/s) and improved for 6MWT (37 vs 48 meters).

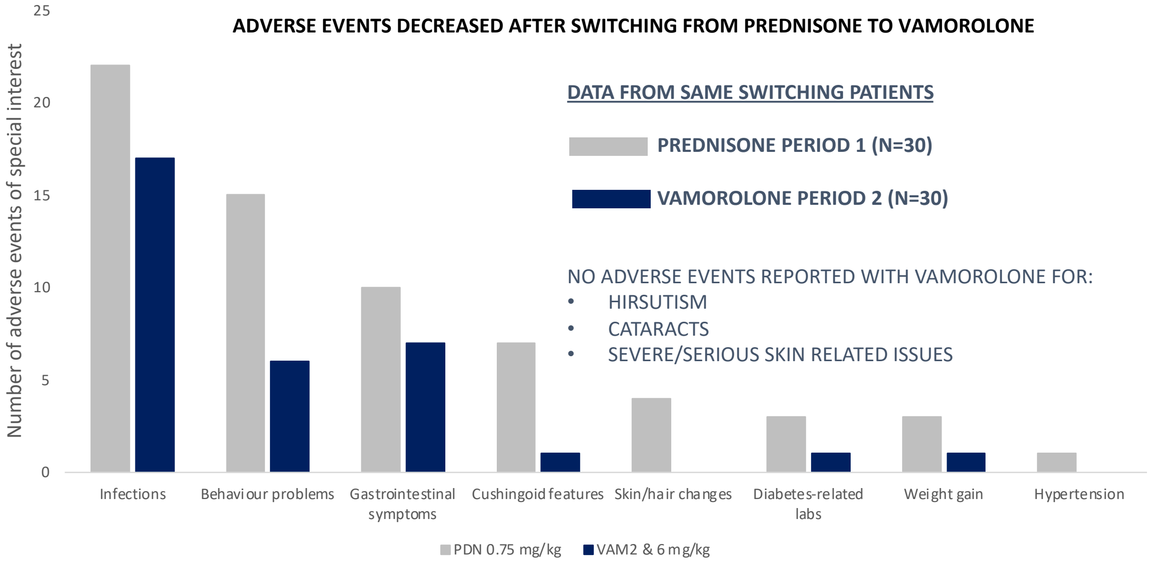
Vamorolone low dose showed clinically relevant and robust efficacy at week 24 for the pre-defined hierarchical endpoints of TTSTAND velocity and 6MWT, as well as NSAA which was an exploratory endpoint. At week 48, vamorolone high dose was statistically superior to vamorolone low dose in TTSTAND velocity (p=0.010) and 6MWT (p=0.047) but not for TTRW velocity (p=0.37) and NSAA (p=0.60).

Vamorolone at both doses was generally safe and well tolerated
For patients who continued the same dose of vamorolone throughout the trial, the safety profile was consistent at week 48 compared to the results previously reported at week 24. Growth velocity was preserved at both vamorolone doses. Body mass index (BMI) was stable for subjects continuing vamorolone low and high dose between assessments at week 24 and week 48 (mean z- score 1.15 vs 1.25 for high dose vamorolone; 0.76 vs 0.91 for low dose vamorolone where a z-score of 0 would represent the median value of an age-matched general population).

Switching from prednisone to vamorolone resulted in multiple positive outcomes
Patients switching from 24-week treatment with prednisone to high-dose vamorolone showed no loss of efficacy through to trial end. In addition, vamorolone treatment at both doses reversed the growth impairment seen during prednisone treatment and was associated with fewer adverse events, including those associated with corticosteroid use.

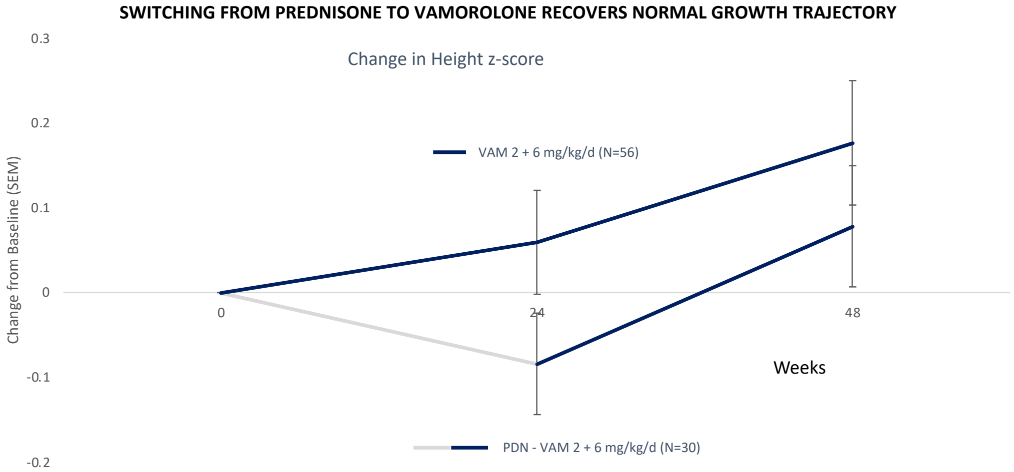
No loss of efficacy seen across multiple outcomes switching to vamorolone
The efficacy benefit seen with prednisone in Period 1 was maintained when subjects were switched to high dose vamorolone during Period 2: TTSTAND velocity (0.28 vs 0.27 rises/s), 6MWT distance (407 vs 408 meters), TTRW velocity (2.20 vs 2.20 m/s) and NSAA (25.6 vs 26.2 points), all absolute values.

Good tolerability and a substantial reduction of side effects
Comparison of the safety outcome parameters of prednisone in Period 1 at week 24, following cross-over to vamorolone low or high dose, showed an improved safety profile. In patients, who were switched from prednisone to high dose vamorolone, the number of total adverse events was reduced by 37% (70 vs 44) and the number of adverse events typically associated with corticosteroids was reduced by 60% (40 vs 16). Of particular interest, the number of adverse events reported as behavioral changes decreased by 60% (15 vs 6). Stunting of growth observed with prednisone was reversed during treatment with high dose vamorolone in Period 2 (mean z-score -0.38 vs -0.12 where a z-score of 0 would represent a normal growth trajectory). An increase of BMI was observed with prednisone in Period 1 (mean z-score from 0.94 to 1.40) but stabilized in Period 2 with high dose vamorolone (mean z-score 1.32). Similar effects for safety and tolerability were observed with the switch from prednisone to low dose vamorolone.



SOURCE: SANTHERA PHARMACEUTICALS

Short stature, behavioral changes and weight gain are some of the important concerns families and patients experience with corticosteroids, often limiting their use or even leading to treatment discontinuation. Based on data from the "VISION-DMD" trial, including promising data on restoration of normal growth, fewer side effects impacting behavior and encouraging data on body mass index (BMI), vamorolone has the potential to emerge as a valuable alternative to corticosteroids, the current standard of care for DMD.

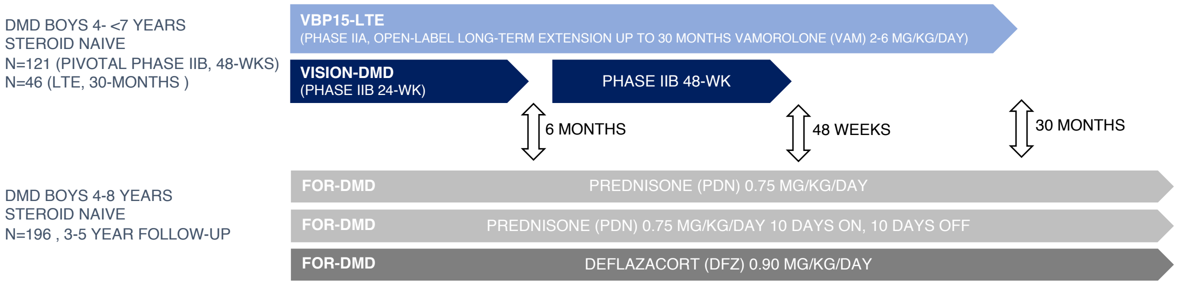


SOURCE: SANTHERA PHARMACEUTICALS

Independent FOR-DMD comparison trial shows comparable efficacy to steroids

Vamorolone's clinical data package for DMD is complemented by the independent "FOR-DMD" Study (n=196) an external, academic-run, double-blind, randomized comparator trial with pre-specified efficacy and safety analysis comparing DMD patients on: 1) vamorolone (Study VBP-15LTE (n=46) 30 months, and VISION-DMD (n=121) Period 1 (24-week) and Period 2 (48-week), and the steroid comparator group (n=196 consisting of 2) prednisone 0.75 mg/kg/day, 3) prednisone 0.75 mg/kg/day 10 days on and 10 days off, and 4) deflazacort 0.90 mg/kg/day (branded Emflaza by PTC Therapeutics in the US).

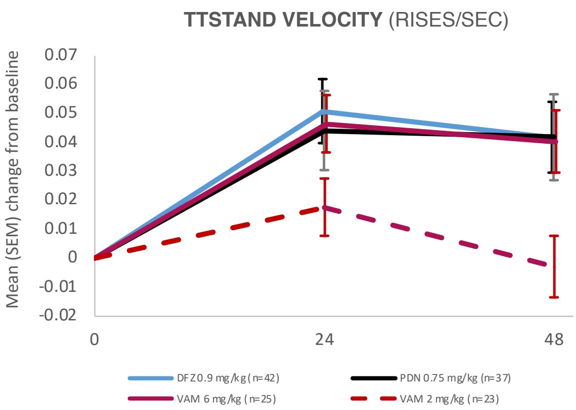
FOR-DMD STUDY AS AN EXTERNAL COMPARATOR

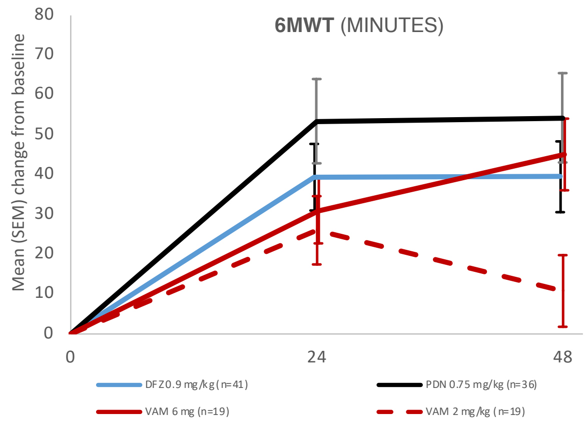


TIME POINT	EFFICACY		SAFETY	
	COMPARISON	METHOD	COMPARISON	METHOD
24 WEEKS / 6 MONTHS	PDN (VISION-DMD) VS. PDN (FOR-DMD)	PROPENSITY SCORE MATCHING	PDN (VISION-DMD) VS. PDN (FOR-DMD)	INCLUSION CRITERIA MATCHING
48 WEEKS / 12 MONTHS	VAM VS. PDN VS. DFZ	PROPENSITY SCORE MATCHING	VAM VS. PDN VS. DFZ	INCLUSION CRITERIA MATCHING
2.5 YEARS	NOT APPLICABLE	NOT APPLICABLE	VAM VS. PDN VS. DFZ	INCLUSION CRITERIA MATCHING

SOURCE: SANTHERA PHARMACEUTICALS

Propensity-matched cross-study comparisons between the VISION-DMD trial pre-specified analyses and the independent FOR-DMD Study external control showed vamorolone high-dose (6 mg/kg/day) had comparable efficacy as conventional steroid therapy in TTSTAND velocity and the 6MWT after 24-week and 48-week treatment. Vamorolone low-dose (2 mg/kg/day) showed less robust efficacy in TTSTAND velocity and the 6MWT after 24-week treatment, with efficacy waning after 48-week treatment.



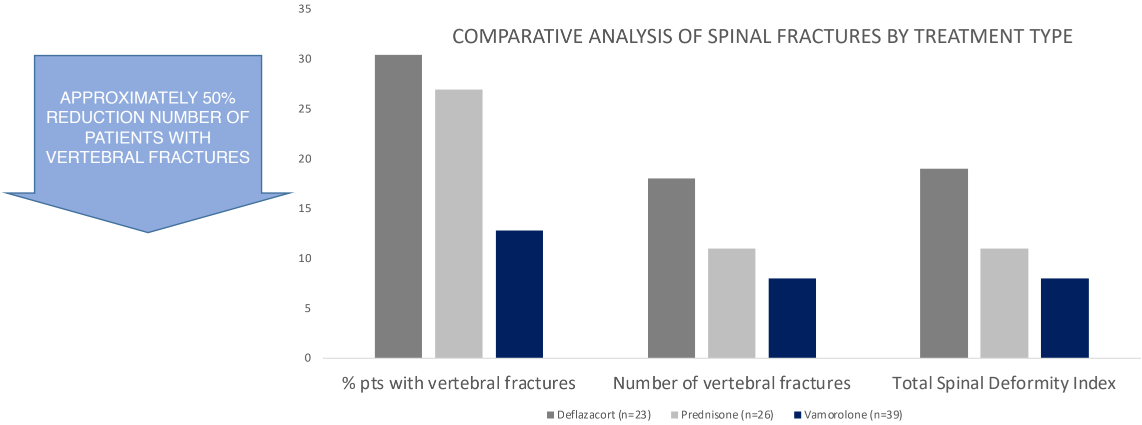


SOURCE: SANTHERA PHARMACEUTICALS

In August 2022, several posters were presented at the World Muscle Society meeting including the poster "The spine fracture burden in boys with DMD treated with the novel dissociative steroid vamorolone versus deflazacort or prednisone" by L.M. Ward *et al.* After 2.5 years of vamorolone treatment, bone turnover markers were not suppressed, bone age delay was minimal and the vertebral (spine) fracture burden appeared to be lower compared to conventional steroids deflazacort and prednisone.

FEWER AND LESS SEVERE SPINAL FRACTURES WITH VAMOROLONE OVER 2.5 YEARS

VAMOROLONE VS. "FOR-DMD", MATCHED COMPARISION, CENTRAL READING USING MODIFIED GENANT

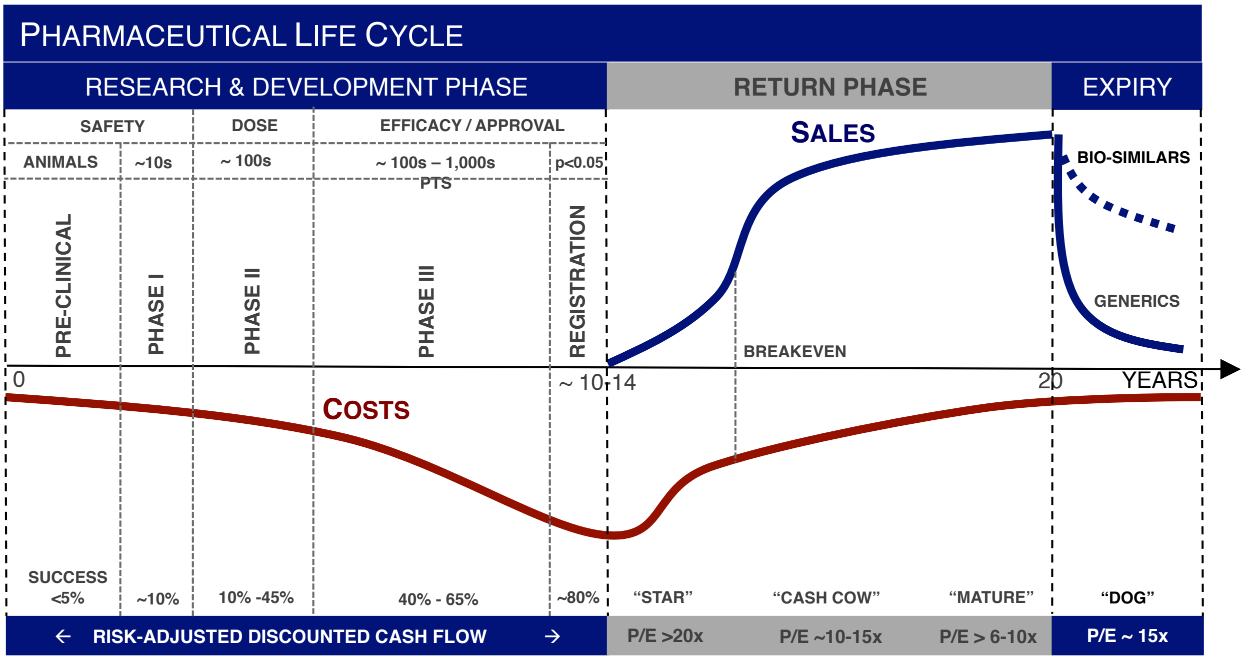


SOURCE: SANTHERA PHARMACEUTICALS

These results suggest that vamorolone may be relatively bone-sparing in ambulatory boys with DMD compared to conventional steroids.

Pharmaceutical life cycle

To determine the value of a prescription (bio)pharmaceutical compound, it is critical to understand its life cycle. Fortunately, all compounds follow the same life cycle. The clock starts ticking after the compound is patented, providing 20 years of protection from generic competition. Market exclusivities can extend this protection period. Additional protection is provided by orphan drug status (10 years in the EU, 7 years in the US). The average Research & Development Phase takes 8-14 years, leading to an effective Return Phase of 6-12 years. The Development Phase has three distinct Phases, focused on safety (Phase I), dose (Phase II) and efficacy/clinical benefit (Phase III). The compound is filed for registration/approval at the FDA (US) or EMA (EU). The Return Phase is characterized by a star, cash cow, and mature phase. After patent expiry (or loss of market exclusivity) generic manufacturers may copycat the branded prescription drug at significantly lower costs, leading to a sales and earnings implosion of the branded drug.



SOURCE: VALUATIONLAB

Success Probabilities & Royalties

In our risk-adjusted NPV calculations, we use standardized success probabilities based on historical clinical success rates. The success rate increases as the project progresses through development. Sales and earnings forecasts are based on the clinical and competitive profile of the compound. The more advanced the compound is, the more accurate the forecasts become as the target market can be defined. We conservatively exclude projects that lack Phase IIa proof-of-concept data in our valuations.

SUCCESS PROBABILITIES & ROYALTIES

DEVELOPMENT STAGE	AIM	WHAT / WHO	SUCCESS PROBABILITY (%)	COSTS (USD MN)	ROYALTIES (%)
PRE-CLINICAL	SAFETY & PHARMACOLOGY DATA	LAB TESTS / ANIMALS - NO HUMANS!	< 5	3	
PHASE I	SCREENING FOR SAFETY	HEALTHY VOLUNTEERS (10'S)	5-15	3	< 5
PHASE IIA	PROOF-OF-CONCEPT	PATIENTS WITH DISEASE (10'S)	10-20		
PHASE II	ESTABLISH THE TESTING PROTOCOL	PATIENTS WITH DISEASE (100'S)	15-35	5	5-15
PHASE IIB	OPTIMAL DOSAGE	PATIENTS WITH DISEASE (100'S)	20-45	5-10	
PHASE III	EVALUATE OVERALL BENEFIT/RISK	PATIENTS WITH DISEASE (1,000'S)	40-65	> 20-1,000	10-25
REGULATORY FILING	DETERMINE PHYSICIAN LABELING	CLINICAL BENEFIT ASSESSMENT	80-90		
APPROVAL	MARKETING AUTHORIZATION	PHYSICIANS FREE TO PRESCRIBE	100		15-30

SOURCE: VALUATIONLAB, TUFTS, FDA, EMA, CLINICALTRIALS.GOV

Important Research Disclosures

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Our financial analyses are based on the "Directives on the Independence of Financial Research" issued by the Swiss Bankers Association in January 2008.

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Risk Qualification

Speculative	less than 1 year cash and breakeven beyond 1 year
High Risk	profitable within 2 years and 1 approved product/key indication (patent expiry > 5 years)
Medium Risk	profitable and/or sales from at least 2 marketed products/key indications (patent expiry > 5 years)
Low Risk	profitable and sales from >2 marketed products/key indications (patent expiry > 5 years)

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