VALUATIONLAB
FINANCIAL ANALYSIS

# SANTHERA PHARMACEUTICALS

**FOCUS AREA: NOVEL THERAPIES IN RARE NEUROMUSCULAR AND PULMONARY (LUNG) DISORDERS**

| KEY DATA | | | SIX: SANN |
|---|---|---|---|
| **MARKET CAPITALIZATION** (CHF MN) | 60 | **SHARE PRICE ON 26 JUNE 2023** | 0.8 |
| **ENTERPRISE VALUE** (CHF MN) | 42 | **RISK-ADJUSTED NPV PER SHARE** (CHF) | 11.2 |
| **ESTIMATED GROSS CASH** * (31 JULY 2023) (CHF MN) | 48 | **UPSIDE/DOWNSIDE** (%) | 1318% |
| **MONTHLY OPERATING EXPENSE** (CHF MN) | 4.8 | **RISK PROFILE** | SPECULATIVE |
| **CASH RUNWAY** * | Q1 2025 | **SUCCESS PROBABILITY LEAD PROJECT** | 80% |
| **BREAK-EVEN** (YEAR) | 2024/2025 | **EMPLOYEES** | 52 |
| **FOUNDED** (YEAR) | 2004 | **LISTED** (YEAR) | 2006 |

| KEY PRODUCTS: | STATUS |
|---|---|
| - VAMOROLONE (DUCHENNE MUSCULAR DYSTROPHY - DMD) | FILED IN US, EU & UK |
| - LONODELESTAT (RARE LUNG DISEASES - TBD) | PHASE II-READY (PAUSED) |
| - RAXONE (LEBER'S HEREDITARY OPTIC NEUROPATHY - LHON) | LAUNCHED (EU ONLY) |

| MAJOR SHAREHOLDERS: | (%) |
|---|---|
| - CATALYST PHARMACEUTICALS * | 18.8 |
| - IDORSIA | 17.7 |
| - EXECUTIVE MANAGEMENT & BOARD | 0.9 |
| **- FREE FLOAT** | **80.3** |
| - AVERAGE DAILY VOLUME (30-DAY) | 617,208 |

| UPCOMING CATALYSTS: | DATE |
|---|---|
| - VAMOROLONE - US APPROVAL FOR DMD (PDUFA DATE) | 26 OCTOBER 2023 |
| - VAMOROLONE - US LAUNCH IN DMD | LATE Q4 2023 |
| - VAMOROLONE - EU & UK APPROVAL FOR DMD | LATE Q4 '23 / EARLY Q1 '24 |

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* ON CLOSING OF THE CATALYST PHARMACEUTICALS LICENSE AGREEMNT IN JULY 2023

ESTIMATES AS OF 26 JUNE 2023

SOURCE: VALUATIONLAB ESTIMATES, SANTHERA PHARMACEUTICALS

# Against all odds

## A much-needed Catalyst – substantial dilution avoided

Santhera is focused on developing and commercializing treatments for rare neuromuscular and lung diseases. The key driver in neuromuscular diseases is vamorolone, a first-in-class dissociative oral steroid, which has been filed for US, EU, and UK approval for treating Duchenne muscular dystrophy (DMD) with approvals expected in Q4 2023. In 2020, Santhera acquired the global rights to vamorolone for all indications from Idorsia (17.7% stakeholder) and ReveraGen, with the potential to monetize geographical rights and non-DMD indications. The key driver in lung diseases is lonodelestat which is phase II-ready for cystic fibrosis (CF) and acute respiratory distress syndrome (ARDS), acquired from Polyphor (now Spexis) in 2018. Vamorolone and lonodelestat target multibillion-dollar markets with high unmet medical need. The anticipated US, EU, and UK approvals and launches of vamorolone in DMD in late 2023/early 2024 signal a new era. The recent Catalyst license agreement for exclusive rights for vamorolone in North America, valued at up to USD 231 mn with significant upfront payments, provides a cash runway into Q1 2025. We derive a sum-of-parts risk-adjusted (r)NPV of CHF 11.6 per share. We qualify Santhera as Speculative pending US, EU, and UK approval of vamorolone in DMD to secure sustainable revenues and profits.

**Key catalysts:**

1) **Vamorolone US approval in DMD – PDUFA date (26 October 2023):** increases our rNPV by CHF 1.4 per share with a 90% success rate, the average of US (100% approved) and EU (80% filed); the US launch by partner Catalyst Pharmaceuticals is targeted for late Q4 2023
2) **Vamorolone EU approval in DMD (Q4 2023):** our rNPV increases by CHF 1.4 per share with a 100% (approved) success rate; launch in the EU Top 5, Austria & Benelux countries by Santhera's own specialist sales force will start in Germany in late Q4 2023 followed by other EU member states in 2024
3) **Vamorolone UK approval in DMD (Q4 2023):** should follow similar timelines as for the EU; potential for early access in the UK and France (with first sales ahead of formal approval likely)

# Flash Update

**Frontloaded license deal with Catalyst for vamorolone rights in North America**
On 20 June 2023, an exclusive license and collaboration agreement was announced with Catalyst Pharmaceuticals (Catalyst) [NASDAQ: CPRX] for the rights of vamorolone, including indications beyond DMD for North America (US, Canada, Mexico) in a deal valued at up to USD 231 mn plus royalties. Catalyst is a commercial-stage US rare disease biopharmaceutical company. The deal is 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 vamorolone should be approved for DMD. We conservatively assume a historical 80% filing success rate.

**Terms of the Catalyst license agreement for vamorolone in North America:**

- Catalyst to commercialize vamorolone in DMD with rights to all potential future indications in North America (US, Canada, Mexico).
- Santhera and Catalyst intend to collaborate on joint development and funding of vamorolone for additional indications beyond DMD.
- Santhera will receive 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 at a price of CHF 0.9477 per share, with a six-month post-closing lock-up and standstill) to support phase IV post-marketing trials in DMD and joint development of additional indications.
- Upon US FDA approval in DMD (26 October 2023 PDUFA date), an additional USD 10 mn milestone payment is due, plus USD 26 mn to pay for approval-related regulatory milestones to third parties (Idorsia and ReveraGen) with 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 vamorolone sales in North America.
- Closing of the transaction is expected in July 2023, subject to customary conditions and regulatory clearances in the US.

Catalyst has an established infrastructure and track record in commercializing rare disease drugs ensuring DMD patients receive vamorolone as quickly as possible in North America. For indications in addition to DMD, Santhera and Catalyst will establish a joint steering committee (JSC) to undertake the joint clinical development of vamorolone for global indications, in which both parties would participate in the development process and funding.

Santhera will continue to focus on the commercialization of vamorolone in DMD in the EU Top 5, Austria, and Benelux countries and will seek partners for commercialization in all other countries. For certain countries where Santhera chooses not to market vamorolone directly, including certain European countries and Japan, it has granted Catalyst a right of first negotiation in partnering discussions.

**Catalyst agreement significantly improves the risk/reward profile for Santhera**

Santhera initially aimed to commercialize vamorolone in DMD through its own specialist sales force in the lucrative US market to maximize the long-term value of its first-in-class dissociative steroid. This would be at the cost of substantial share dilution to raise sufficient cash to build up a US and European specialist sales force for vamorolone in the current adverse financial markets. If unsuccessful, the company would be at risk of running out of cash at year-end – the critical time to launch vamorolone in DMD - with a stretched balance sheet.

In our view, the Catalyst licensing agreement has significantly improved the risk/reward profile for Santhera and, based on our rNPV per share calculation, continues to provide significant equity upside – a multiple from the current depressed share price – but without substantial share dilution, the risk of running out of cash and a cash runway guided into Q1 2025 (from Q4 2023), with a massively de-risked balance sheet as the proceeds of the deal allow for the repayment of all short-term debt with Highbridge.

Santhera assessed over a dozen proposals leading to a short list of three final candidates for a strategic partnership. The NPV of the Catalyst partnership agreement prevailed, providing a partner with an established commercial infrastructure for selling rare disease drugs such as vamorolone and motivated to develop the compound in other (potentially larger) indications than DMD.

**Impact of the Catalyst licensing agreement:**

- **Commercialization North America:** Catalyst Pharmaceuticals is an experienced rare disease specialist with the marketing muscle to commercialize vamorolone in DMD successfully with the willingness and sufficient funds to invest in other indications outside DMD.
- **Commercialization Europe and ROW:** Santhera retains the rights for Europe and the Rest of the World (ROW). The agreement provides non-dilutive cash for Santhera to build up a sales infrastructure for the EU Top 5 and Benelux countries, while the rights of other regions can be monetized. Last year, the rights to vamorolone in the Greater China Region were licensed to Sperogenix in a deal valued at up to USD 124 mn.
- **Pipeline:** The agreement accelerates the development of vamorolone in indications outside DMD, which could potentially top vamorolone sales in DMD. Catalyst has sufficient cash to explore multiple potential indications outside DMD at once. Without this deal, Santhera would initially have limited cash resources to quickly develop vamorolone in other indications. The incremental value of these indications with a patent life of up to 2040 in the US could be substantial. Note that we conservatively exclude any forecasts for vamorolone outside DMD.
- **Frontloaded licensing agreement:** The Catalyst agreement is frontloaded with USD 126 mn of the up to USD 231 mn deal value to be paid this year. USD 90 mn will be paid on closing of the transaction in July, months ahead of the likely US approval of vamorolone. This indicates that Catalyst is confident that vamorolone will be approved for DMD in the US. We conservatively assume an 80% (filing) success rate.
- **Balance sheet:** The upfront proceeds allow for the repayment of all short-term debt with Highbridge and the overall strengthening and de-risking of the balance sheet.
- **Cash position and cash runway:** On closing of the transaction, cash and cash equivalents are estimated to amount to approximately CHF 48 mn (assuming the USD 90 mn (CHF 81 mn) milestone and the repayment of approximately CHF 33 mn short-

term debt). Santhera guides that its cash runway will be extended into Q1 2025 (from Q4 2023).

- **Breakeven:** Based on our forecasts, Santhera should breakeven this year and even report a profit on the high upfront payments. Development of, for instance, lonodelestat in cystic fibrosis or acute respiratory distress syndrome (ARDS) and the repayment of the convertible bonds of CHF 30 mn in 2024 could lead to a temporary loss in that year.
- **Risk-adjusted net present value (rNPV):** We calculate that the rNPV per share of the Catalyst agreement for the rights of vamorolone in North America is still worth a multiple of Santhera's current share price and market capitalization. The USD 90 mn upfront payment alone is already higher than the current CHF 60 mn (USD 67 mn) market capitalization of Santhera. In our view, the rNPV per share represents the best way to compare the impact of Catalyst commercializing vamorolone in North America and Santhera building its own US sales force. The rNPV per share includes the impact of the substantial share dilution to raise capital to fund the build-up of a US sales infrastructure. For the Catalyst agreement, we calculate an rNPV of CHF 11.6 per share for vamorolone in DMD (see below). This is 20% lower than our rNPV of CHF 14.5 per share in our last Santhera Valuation Report in December 2022, which included a 65% share dilution to raise CHF 40 mn to pay for a US and EU specialist sales force. Our previous report anticipated Priority Review with US approval expected four months earlier. The costs of these additional months have not been accounted for in this calculation.

**DMD peak sales of CHF 700 mn - rNPV of CHF 11.6 per share**

We forecast global peak sales of CHF 700 mn for vamorolone in DMD alone, assuming first launches in the US and the EU in late 2023/early 2024 and in the Greater China Region in 2024. Vamorolone has extensive protection and market exclusivity 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 until 2035.

We conservatively assume an annual weight-based treatment cost in the US at a ~15% premium to PTC Therapeutics' Emflaza (deflazacort) of USD 58,625 for patients weighing ~34 kg (aged 4-11 years) and up to USD 109,375 for patients weighing ~63 kg (aged 11-22 years). In the EU and ROW, we apply a ~40% discount to the US pricing, while in China, we apply an 80% discount. In patients aged 4-11 years, we assume peak penetration rates of up to 45% (US) and half the amount (23%) in patients aged 11-22 as the disease progresses.

In the US, our forecasts largely reflect the Catalyst agreement, including a USD 75 mn upfront cash payment on the closing of the transaction and an additional USD 10 mn payment on US approval of vamorolone in DMD next to USD 26 mn to pay for approval-related regulatory milestones to third parties (Idorsia and ReveraGen) and additional sales milestones of CHF 89 mn (eligible for up to USD 105 mn sales milestones). We assume tiered net sales royalties starting at 7% and gradually increasing to 12%. Catalyst pays for third-party royalty obligations (assumed tiered single to low double-digit percentage royalties to Idorsia/ReveraGen).

In the EU, we account for 10% COGS, the buildup of a small specialist sales force in the EU Top 5, Austria, and Benelux countries, and tiered single to low double-digit percentage sales royalty payments to Idorsia and ReveraGen.

In the Greater China Region, our forecasts largely reflect the Sperogenix agreement with conservatively USD 50 mn milestones (eligible up to USD 124 mn for all rare disease indications) and 15% sales royalties.

In the ROW, we assume Santhera to partner vamorolone in return for CHF 31 mn milestones and 15% sales royalties from its partner(s).

Our rNPV amounts to CHF 873 mn or CHF 11.6 per share, applying a historical 80% (filing) success rate and a WACC of 7% (for details, see the following page).

# Forecasts & Sensitivity Analysis

## VAMOROLONE - FINANCIAL FORECASTS FOR DUCHENNE MUSCULAR DYSTROPHY

| | |
|---|---|
| **INDICATION** | PRESERVATION OF MUSCLE FUNCTION IN DMD WITH IMPROVED SAFETY AND TOLERABILITY PROFILE THAN EXISTING STEROIDS |
| **DOSAGE** | TO BE DETERMINED (2 MG UP TO 6 MG/KG/DAY) |
| **PRICE** | ANNUAL COST PER KG WEIGHT: US: USD 1,750 (~15% PREMIUM TO EMFLAZA); EU: EUR 900; CHINA: USD 350; ROW: USD 1,000; CONSERVATIVE PRICING TO FACILITATE REIMBURSEMENT |
| **STANDARD OF CARE** | STEROIDS (OFF-LABEL) & BRANDED 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: US 7-YEAR; EU: 10-YEAR + 2-YEAR PEDIATRIC; US POLYMORPH PATENT: 2040 (NEW) |
| **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 (FAST TRACK) - US: PDUFA 26 OCT 2022, LAUNCH Q4 2023; EU: FILING SEP 2022, APPROVAL END 2023; LAUNCH Q1 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) | 2022E | 2023E | 2024E | 2025E | 2026E | 2027E | 2028E | 2029E | 2030E | 2031E | 2032E |
|---|---|---|---|---|---|---|---|---|---|---|---|
| NUMBER OF PATIENTS | 16,895 | 17,233 | 17,577 | 17,929 | 18,287 | 18,653 | 19,026 | 19,407 | 19,795 | 20,191 | 20,595 |
| PATIENTS AGE 4-11 YEARS (~32%) | 5,406 | 5,514 | 5,625 | 5,737 | 5,852 | 5,969 | 6,088 | 6,210 | 6,334 | 6,461 | 6,590 |
| PENETRATION (%) | 0% | 0% | 20% | 30% | 38% | 42% | 44% | 45% | 45% | 45% | 45% |
| TREATED PATIENTS (AGE 4-11 YEARS) | 0 | 0 | 1,125 | 1,721 | 2,224 | 2,507 | 2,679 | 2,795 | 2,850 | 2,907 | 2,966 |
| ANNUAL COST OF THERAPY (AVE. ~34 KG) (CHF) | 55,811 | 52,847 | 52,348 | 52,348 | 52,348 | 52,348 | 52,348 | 52,348 | 52,348 | 52,348 | 52,348 |
| **SALES PATIENTS AGE 4-11 YEARS (CHF MN)** | **0** | **0** | **59** | **90** | **116** | **131** | **140** | **146** | **149** | **152** | **155** |
| PATIENTS AGE 12-22 YEARS (~44%) | 7,941 | 8,099 | 8,261 | 8,427 | 8,595 | 8,767 | 8,942 | 9,121 | 9,304 | 9,490 | 9,679 |
| PENETRATION (%) | 0% | 0% | 0% | 0% | 10% | 15% | 19% | 21% | 22% | 23% | 23% |
| TREATED PATIENTS (AGE 12-22 YEARS) | 0 | 0 | 0 | 0 | 860 | 1,315 | 1,699 | 1,915 | 2,047 | 2,135 | 2,178 |
| ANNUAL COST OF THERAPY (AVE. ~63 KG) (CHF) | 104,125 | 98,596 | 97,664 | 97,664 | 97,664 | 97,664 | 97,664 | 97,664 | 97,664 | 97,664 | 97,664 |
| **SALES PATIENTS AGE 12-22 YEARS (CHF MN)** | **0** | **0** | **0** | **0** | **84** | **128** | **166** | **187** | **200** | **209** | **213** |
| **SALES (CHF MN)** | **0** | **0** | **59** | **90** | **200** | **260** | **306** | **333** | **349** | **361** | **368** |
| CHANGE (%) | | | | 53% | 122% | 30% | 18% | 9% | 5% | 3% | 2% |
| ROYALTIES (%) | 0% | 7% | 7% | 9% | 9% | 11% | 11% | 12% | 12% | 12% | 12% |
| ROYALTY INCOME (CHF MN) | 0 | 0 | 4 | 8 | 18 | 29 | 34 | 40 | 42 | 43 | 44 |
| UPFRONT AND MILESTONE INCOME (CHF MN) | 0 | 100 | 0 | 9 | 18 | 0 | 27 | 0 | 0 | 36 | 0 |
| MILESTONE PAYMENTS TO IDORSIA/REVERAGEN (CHF MN) | 0 | -23 | 0 | -18 | 0 | 0 | 0 | 0 | 0 | 0 | 0 |
| M&S COSTS (CHF MN) | -9 | -6 | 0 | 0 | 0 | 0 | 0 | 0 | 0 | 0 | 0 |
| **PROFIT BEFORE TAX (CHF MN)** | **-9** | **70** | **4** | **-1** | **36** | **29** | **60** | **40** | **42** | **79** | **44** |
| TAXES (CHF MN) | 0 | 0 | 0 | 0 | -2 | -4 | -12 | -8 | -8 | -16 | -9 |
| **PROFIT (CHF MN)** | **-9** | **70** | **4** | **-1** | **34** | **24** | **48** | **32** | **34** | **63** | **35** |

| EUROPE TOP 5, AUSTRIA + BENELUX (SANTHERA SPECIALIST SALES FORCE) | 2022E | 2023E | 2024E | 2025E | 2026E | 2027E | 2028E | 2029E | 2030E | 2031E | 2032E |
|---|---|---|---|---|---|---|---|---|---|---|---|
| NUMBER OF PATIENTS | 18,434 | 18,619 | 18,805 | 18,993 | 19,183 | 19,375 | 19,568 | 19,764 | 19,962 | 20,161 | 20,363 |
| GROWTH (%) | 1% | 1% | 1% | 1% | 1% | 1% | 1% | 1% | 1% | 1% | 1% |
| PATIENTS AGE 4-11 YEARS (~32%) | 5,899 | 5,958 | 6,018 | 6,078 | 6,138 | 6,200 | 6,262 | 6,324 | 6,388 | 6,452 | 6,516 |
| PENETRATION (%) | 0% | 0.0% | 9% | 17% | 31% | 39% | 41% | 43% | 44% | 45% | 45% |
| TREATED PATIENTS (AGE 4-11 YEARS) | 0 | 0 | 511 | 1,033 | 1,903 | 2,418 | 2,567 | 2,720 | 2,811 | 2,903 | 2,932 |
| ANNUAL COST OF THERAPY (AVE. ~34 KG) (CHF) | 30,892 | 29,730 | 29,621 | 29,621 | 29,621 | 29,621 | 29,621 | 29,621 | 29,621 | 29,621 | 29,621 |
| **SALES PATIENTS AGE 4-11 YEARS (CHF MN)** | **0** | **0** | **15** | **31** | **56** | **72** | **76** | **81** | **83** | **86** | **87** |
| PATIENTS AGE 12-22 YEARS (~44%) | 8,111 | 8,192 | 8,274 | 8,357 | 8,440 | 8,525 | 8,610 | 8,696 | 8,783 | 8,871 | 8,960 |
| PENETRATION (%) | 0% | 0% | 0% | 4% | 9% | 16% | 20% | 21% | 22% | 22% | 23% |
| TREATED PATIENTS (AGE 12-22 YEARS) | 0 | 0 | 0 | 355 | 717 | 1,321 | 1,679 | 1,783 | 1,888 | 1,952 | 2,016 |
| ANNUAL COST OF THERAPY (AVE. 63 KG) (CHF) | 57,635 | 55,467 | 55,262 | 55,262 | 55,262 | 55,262 | 55,262 | 55,262 | 55,262 | 55,262 | 55,262 |
| **SALES PATIENTS AGE 12-22 YEARS (CHF MN)** | **0** | **0** | **0** | **20** | **40** | **73** | **93** | **99** | **104** | **108** | **111** |
| **SALES (CHF MN)** | **0** | **0** | **15** | **50** | **96** | **145** | **169** | **179** | **188** | **194** | **198** |
| CHANGE (%) | | | | 232% | 91% | 51% | 17% | 6% | 5% | 3% | 2% |
| COGS (10%) (CHF MN) | 0 | 0 | -2 | -5 | -10 | -14 | -17 | -18 | -19 | -19 | -20 |
| TIERED ROYALTY PAYMENTS TO REVERAGEN (%) | 5% | 5% | 5% | 5% | 5% | 5% | 7% | 7% | 7% | 7% | 7% |
| TIERED ROYALTY PAYMENTS TO REVERAGEN (CHF MN) | 0 | 0 | -1 | -3 | -5 | -7 | -11 | -12 | -12 | -13 | -13 |
| R&D COSTS (EXCL. IDORSIA/REVERAGEN PAYMENTS) (CHF MN) | -24 | -5 | -9 | -7 | -5 | -2 | 0 | 0 | 0 | 0 | 0 |
| M&S COSTS (CHF MN) | -2 | -8 | -11 | -13 | -13 | -17 | -20 | -21 | -23 | -23 | -24 |
| **PROFIT BEFORE TAX (CHF MN)** | **-26** | **-13** | **-7** | **23** | **63** | **104** | **121** | **128** | **134** | **139** | **142** |
| TAXES (CHF MN) | 0 | 0 | 0 | 0 | -3 | -16 | -24 | -26 | -27 | -28 | -28 |
| **PROFIT (CHF MN)** | **-26** | **-13** | **-7** | **23** | **60** | **88** | **97** | **102** | **107** | **111** | **113** |

| GREATER CHINA REGION (SPEROGENIX) | 2022E | 2023E | 2024E | 2025E | 2026E | 2027E | 2028E | 2029E | 2030E | 2031E | 2032E |
|---|---|---|---|---|---|---|---|---|---|---|---|
| NUMBER OF PATIENTS | 58,317 | 58,901 | 59,490 | 60,084 | 60,685 | 61,292 | 61,905 | 62,524 | 63,149 | 63,781 | 64,419 |
| TREATED PATIENTS (AGE 4-11 YEARS) | 0 | 0 | 0 | 135 | 874 | 1,569 | 2,397 | 3,121 | 3,678 | 4,000 | 3,463 |
| ANNUAL COST OF THERAPY (AVE. ~34 KG) (CHF) | | | 10,470 | 10,470 | 10,470 | 10,470 | 10,470 | 10,470 | 10,470 | 10,470 | 10,470 |
| **SALES PATIENTS AGE 4-11 YEARS (CHF MN)** | | **0** | **0** | **1** | **9** | **16** | **25** | **33** | **39** | **42** | **36** |
| TREATED PATIENTS (AGE 12-22 YEARS) | 0 | 0 | 0 | 0 | 0 | 144 | 800 | 1,411 | 2,122 | 2,728 | 3,179 |
| ANNUAL COST OF THERAPY (AVE. ~63 KG) (CHF) | | | 19,533 | 19,533 | 19,533 | 19,533 | 19,533 | 19,533 | 19,533 | 19,533 | 19,533 |
| **SALES PATIENTS AGE 12-22 YEARS (CHF MN)** | | **0** | **0** | **0** | **0** | **3** | **16** | **28** | **41** | **53** | **62** |
| **SALES (CHF MN)** | **0** | **0** | **0** | **1** | **9** | **19** | **41** | **60** | **80** | **95** | **98** |
| CHANGE (%) | | | | | | | | | | | |
| ROYALTY INCOME (15%) (CHF MN) | 0 | 0 | 0 | 0 | 1 | 3 | 6 | 9 | 12 | 14 | 15 |
| UPFRONT AND MILESTONE INCOME (CHF MN) | 11.190 | 7 | 0 | 4 | 0 | 0 | 0 | 4 | 0 | 9 | 0 |
| TIERED ROYALTY PAYMENTS TO REVERAGEN (CHF MN) | 0 | 0 | 0 | 0 | 0 | 0 | 0 | 0 | 0 | 0 | 0 |
| MANUFACTURING INCOME (12%) (CHF MN) | 0 | 0 | 0 | 0 | 1 | 2 | 5 | 7 | 10 | 11 | 12 |
| COGS (10%) (CHF MN) | 0 | 0 | 0 | 0 | -1 | -2 | -4 | -6 | -8 | -10 | -10 |
| **PROFIT BEFORE TAX (CHF MN)** | **11** | **7** | **0** | **5** | **2** | **3** | **7** | **15** | **14** | **25** | **17** |
| TAXES (CHF MN) | 0 | 0 | 0 | 0 | 0 | 0 | -1 | -3 | -3 | -5 | -3 |
| **PROFIT (CHF MN)** | **11** | **7** | **0** | **5** | **1** | **3** | **6** | **12** | **11** | **20** | **13** |

| REST OF WORLD (JAPAN & AUSTRALIA) (PARTNERS) | 2022E | 2023E | 2024E | 2025E | 2026E | 2027E | 2028E | 2029E | 2030E | 2031E | 2032E |
|---|---|---|---|---|---|---|---|---|---|---|---|
| NUMBER OF PATIENTS | 5,120 | 5,171 | 5,223 | 5,275 | 5,328 | 5,381 | 5,435 | 5,490 | 5,544 | 5,600 | 5,656 |
| TREATED PATIENTS (AGE 4-11 YEARS) | 0 | 0 | 0 | 34 | 170 | 276 | 383 | 457 | 497 | 538 | 543 |
| ANNUAL COST OF THERAPY (AVE. ~34 KG) (CHF) | | | 29,913 | 29,913 | 29,913 | 29,913 | 29,913 | 29,913 | 29,913 | 29,913 | 29,913 |
| **SALES PATIENTS AGE 4-11 YEARS (CHF MN)** | | **0** | **0** | **1** | **5** | **8** | **11** | **14** | **15** | **16** | **16** |
| TREATED PATIENTS (AGE 12-22 YEARS) | 0 | 0 | 0 | 0 | 0 | 25 | 128 | 206 | 287 | 342 | 372 |
| ANNUAL COST OF THERAPY (AVE. ~63 KG) (CHF) | | | 55,808 | 55,808 | 55,808 | 55,808 | 55,808 | 55,808 | 55,808 | 55,808 | 55,808 |
| **SALES PATIENTS AGE 12-22 YEARS (CHF MN)** | | **0** | **0** | **0** | **0** | **1** | **7** | **12** | **16** | **19** | **21** |
| **SALES (CHF MN)** | **0** | **0** | **0** | **1** | **5** | **10** | **19** | **25** | **31** | **35** | **37** |
| CHANGE (%) | | | | | | | | | | | |
| ROYALTY INCOME (15%) (CHF MN) | 0 | 0 | 0 | 0 | 1 | 1 | 3 | 4 | 5 | 5 | 6 |
| UPFRONT AND MILESTONE INCOME (CHF MN) | 0 | 0 | 4 | 13 | 0 | 9 | 0 | 0 | 2 | 3 | 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** | **4** | **14** | **1** | **10** | **3** | **4** | **6** | **8** | **6** |
| TAXES (CHF MN) | 0 | 0 | 0 | 0 | 0 | -2 | -1 | -1 | -1 | -2 | -1 |
| **PROFIT (CHF MN)** | **0** | **0** | **4** | **14** | **1** | **9** | **2** | **3** | **5** | **6** | **4** |

| | 2022E | 2023E | 2024E | 2025E | 2026E | 2027E | 2028E | 2029E | 2030E | 2031E | 2032E |
|---|---|---|---|---|---|---|---|---|---|---|---|
| **GLOBAL PRODUCT SALES (CHF MN)** | **0** | **0** | **74** | **143** | **311** | **433** | **534** | **598** | **648** | **685** | **702** |
| CHANGE (%) | | | | 93% | 118% | 39% | 23% | 12% | 8% | 6% | 2% |
| **GLOBAL PROFIT (CHF MN)** | **-24** | **65** | **2** | **40** | **96** | **124** | **153** | **149** | **157** | **201** | **167** |
| CHANGE (%) | -5% | -371% | -97% | 2282% | 140% | 29% | 23% | -2% | 5% | 28% | -17% |

| | |
|---|---|
| WACC (%) | 7% |
| **NPV TOTAL PROFIT (CHF MN)** | **1,091** |
| NUMBER OF SHARES (MN) | 75.3 |
| **NPV PER SHARE (CHF)** | **14** |
| SUCCESS PROBABILITY | 80% (= FILED) |
| **RISK ADJUSTED NPV PER SHARE (CHF)** | **11.6** |

### SENSITIVITY ANALYSIS

| | | WACC (%) | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | CHF/SHARE | 5.5 | 6.0 | 6.5 | 7.0 | 7.5 | 8.0 | 8.5 |
| | 100% | 16.2 | 15.6 | 15.0 | 14.5 | 14.0 | 13.5 | 13.0 |
| | 90% | 14.6 | 14.0 | 13.5 | 13.0 | 12.6 | 12.1 | 11.7 |
| | 80% | 13.0 | 12.5 | 12.0 | **11.6** | 11.2 | 10.8 | 10.4 |
| **SUCCESS PROBABILITY** | 70% | 11.3 | 10.9 | 10.5 | 10.1 | 9.8 | 9.4 | 9.1 |
| | 65% | 10.5 | 10.1 | 9.8 | 9.4 | 9.1 | 8.8 | 8.4 |
| | 50% | 8.1 | 7.8 | 7.5 | 7.2 | 7.0 | 6.7 | 6.5 |

ESTIMATES AS OF 26 JUNE 2023

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. Vamorolone 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 potential 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. Santhera has filed in the US, EU, and UK and awaits approval (US: 26 October 2023 PDUFA date) and launch in these major markets in late 2023/early 2024.

**Pathway:** Vamorolone enjoys in both the EU and US orphan drug designation, an incentive to develop drugs for rare diseases.

**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:** Vamorolone can potentially 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. Vamorolone slows the progressive loss of muscle function and the loss of mobility. Vamorolone 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, the superior safety and tolerability profile should lead to fewer secondary complications such as bone fractures and increased patient compliance and long-term treatment outcomes.

**Partner:** In North America, vamorolone will be commercialized by Catalyst with near-term milestone payments of USD 126 mn, up to USD 105 mn additional sales milestones, and up to low-teen percentage royalties on sales. Santhera plans to sell vamorolone in DMD through its own small specialist sales force in the EU Top 5 and Benelux countries that it will build up shortly after approval and seek commercialization partners in ROW similar to the Sperogenix agreement for the Greater China region. Santhera will seek develop and commercialization partners for vamorolone in other indications outside DMD, such as asthma, chronic obstructive pulmonary disease (COPD), ulcerative colitis and inflammatory bowel disease (IBD), among others. Peak sales in these indications could be a multiple of vamorolone in DMD and other rare neuromuscular diseases.

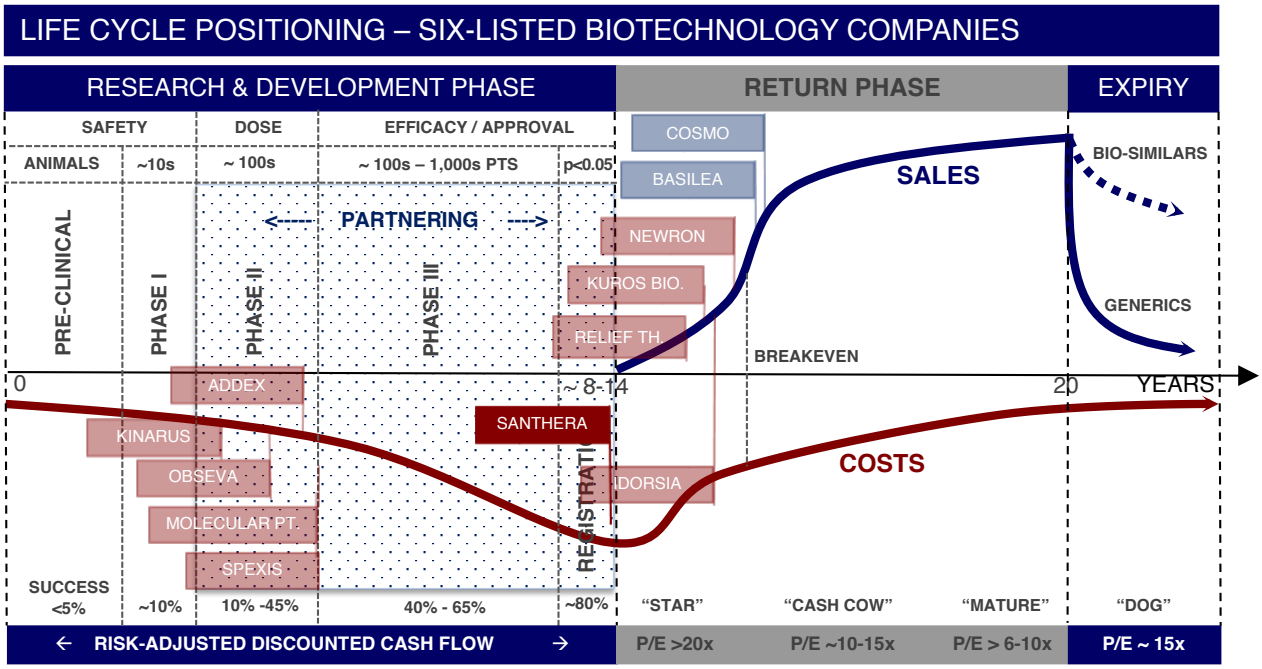
# Investment Case, Strategy & Cash

## Investment case in a nutshell

Against all odds (e.g., the termination of Puldysa in DMD triggering a corporate restructuring, successive funding shortages, vamorolone filing delay due to a third-party manufacturer, the COVID-19 pandemic, and adverse financial markets), Santhera is awaiting approval of vamorolone to treat DMD in the US, EU, and UK with approvals in Q4 2023 and launches in late Q4 2023/early Q1 2024. Despite an excellent licensing agreement with Catalyst for vamorolone in North America, avoiding substantial share dilution and cash burn to build up a US sales infrastructure, Santhera's share price continues to drop. We believe the stock has been largely de-risked with a historical 80% (filing) success rate. We expect a sharp rebound by a multiple of the current low share price upon US, EU, and UK approval and launch. Vamorolone targets peak sales of CHF 700 mn with the potential to become a foundational DMD treatment, which can be used alone or in combination with approved DMD drugs and new gene therapies such as Sarepta's recently approved Elevidys. Based on our detailed bottom-up forecasts for Santhera's sole key driver, vamorolone in DMD with ample patent life and market exclusivity, we calculate a sum-of-the-parts risk-adjusted NPV of CHF 845 mn or CHF 11.2 per share with a cash runway into Q1 2025 providing equity upside of 1,318% from the current depressed share price.

## Life Cycle Positioning – Speculative

We qualify Santhera's risk profile as Speculative, with no sustainable product revenues and profits yet. In our view, the likely US, EU, and UK approvals of vamorolone in DMD in late 2023 and successive launches in late Q4 2023/early Q1 2024 will be transformational for Santhera, leading to a substantial re-rating (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 pulmonary (lung) 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. 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, with a North American headquarters in Burlington, Massachusetts, USA. Santhera has 52 employees globally but expects to increase that number to build up its specialist sales force ahead of the anticipated EU launch of its key drug, vamorolone in Duchenne muscular dystrophy (DMD), in late 2023.

**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 in rare diseases with a special focus on two therapeutic areas:

1. **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:

    - **Vamorolone** for the treatment of DMD patients with early-to-mid-stage disease; positive top line results pivotal "VISION-DMD" trial reported in June 2021 (24-week data) and November 2021 (48-week data); 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; supported by open-label "Study VBP-15 LTE" long-term extension trial and independent double-blinded, randomized FOR-DMD steroid comparator trial; filing completed in the EU (September 2022) and US (October 2022); US approval expected on 26 October 2023 and launch by North America partner Catalyst in Q4 2023 with USD 126 mn upfront milestones, up to USD 105 mn sales milestones and tiered up to low-teen percentage royalties on net sales (with Catalyst to pay third-party royalty obligations in North America); EMA CHMP opinion expected in Q3 2023 followed by EU approval 67 days later, first EU launch in Germany in late Q4 2023 followed by other member states in early 2024; China launch in 2025 by commercialization partner Sperogenix with up to USD 124 mn upfront, regulatory and commercial milestones and double-digit royalties on net sales in

the Greater China Region; we forecast global peak sales of around CHF 700 mn in DMD alone; potential for out licensing in geographies outside the US and EU Top 5 and Benelux countries or 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; acquired the global rights for all indications from Idorsia and originator ReveraGen in September 2020; Idorsia has 17.7% stake in Santhera; upon closing of the transaction, Catalyst will acquire an 18.8% stake in Santhera with a 6-month lock-up period.

2. **Pulmonary (lung) diseases:** treatments for diseases that affect the airway, lung tissue, or lung circulation leading to difficulty in breathing, infection, inflammation, or heart/lung complications; Santhera's key pulmonary drug includes:

    - **Lonodelestat** for the treatment of neutrophilic lung diseases, including cystic fibrosis (CF) and acute respiratory distress syndrome (ARDS); clinical development has been paused until sufficient funds have been secured; positive results phase Ib multiple ascending dose (MAD) trial in cystic fibrosis reported in March 2021; phase II-ready for proof-of-concept (POC) trials in ARDS or cystic fibrosis targeting multi-billion-dollar markets; peak sales to be determined upon start of POC; potential in other lung disorders associated with high hNE activity, including AAT (alpha-1 antitrypsin deficiency), NCFB (non-cystic fibrosis bronchiectasis) and PCD (primary ciliary dyskinesia) to be collaborated and explored with experts; global rights acquired from Polyphor (now Spexis) in 2018

**Sales of Raxone in LHON phasing out after the transfer of the business to Chiesi**

Santhera has enjoyed commercial success with Raxone (idebenone) in the rare neuro-ophthalmological disease Leber's Hereditary Optic Neuropathy (LHON). This ultra-rare genetic eye disease leads to sudden blindness, with a 1 in a million incidence. Raxone was initially commercialized largely by Santhera's own specialist sales force in the EU. In May 2019, the company entered into an exclusive license agreement with the Italian private pharmaceutical company Chiesi Group for the global rights (excluding North America and France) of Raxone in LHON and all other ophthalmological indications for a total consideration of up to CHF 105 mn of which CHF 46.4 mn was recognized as revenue in 2019. This marked Santhera's exit from neuro-ophthalmological diseases, the company's first therapeutic area with commercial success.

In FY 2022, Santhera reported revenue from contracts with customers (mainly France) of CHF 6.3 mn (FY 2021: CHF 4.5 mn). Net sales amounted to CHF -5.9 mn (FY 2021: CHF 2.9 mn). The negative sales are attributable to an additional CHF 6.0 mn that has been accrued and offset against sales in the context of ongoing reimbursement negotiations in France. Since its launch in 2018, Raxone was reimbursed in France under a temporary financing scheme. From August 2021, when Raxone was removed from the list of reimbursed drugs, Santhera agreed to supply Raxone free of charge.

Reimbursement discussions have now concluded with an agreement on Raxone pricing. Raxone will now be on the list of reimbursed products in France and resume sales. The new price for Raxone is lower than the price applied under the temporary pricing scheme, leading to a settlement payment, as announced earlier. Santhera will make repayments in the total amount of approximately EUR 25 mn, with 30% due around mid-2024 and the

remainder one year later. Accruals for such payments have already been made. The first payment of around EUR 7.5 mn is expected to be covered by sales generated until mid-2024, while the majority of the second payment of around CHF 17.5 mn will be covered by sales beyond mid-2025. Santhera is entitled to up to EUR 49 mn additional sales milestone payments upon Chiesi reaching certain sales targets. We conservatively expect a CHF 15 mn sales milestone from Chiesi at the earliest in 2024. The settlement enables Santhera to advance discussions on completing the global out-licensing of Raxone, which is further supported by encouraging clinical data with Raxone from trials completed in the recent past, including the long-term "LEROS" phase IV trial that met its primary endpoint with high statistical evidence.

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

- **US and EU approval of vamorolone in DMD based on the positive "VISION-DMD" trial:** The positive "VISION-DMD" 24-week (Period 1) reached its primary endpoint, showing a clinically relevant and highly statistically significant (p=0.002) increase in the time-to-stand (TTSTAND) velocity of 0.06 rises per second with the high-dose (6 mg/kg/day) of vamorolone compared to placebo. Four out of five secondary endpoints and two additional exploratory endpoints were reached with high statistical significance. Additionally, the trial showed positive 48-week data (Period 2) required for EU filing. The "VISION-DMD" trial was developed under FDA and EMA scientific advice and is considered a pivotal trial for US approval. The FDA set a 26 October 2023 Prescription Drug User Fee Act (PDUFA) target date when it expects to complete its review. The US launch should occur a quarter later by Catalyst, Santhera's new commercialization partner for North America. The EMA's CHMP is expected to issue an opinion in late Q3 2023, with EU approval to follow 67 days later and the first launch in Germany by Santhera's specialist sales force in late 2023, followed by other member states in early 2024.

- **Implement the Catalyst agreement:** Successfully close the transaction and cooperate with Catalyst to prepare for US approval and launch of vamorolone in DMD and set up a Joint Steering Committee for development of vamorolone in additional indications.

- **Build up EU commercial infrastructure:** Intensify the pre-commercialization activities in anticipation of the upcoming EU approval and launch of vamorolone and build a specialist sales infrastructure for the EU Top 5, Austria, and Benelux countries.

- **Prepare for a POC trial of lonodelestat in ARDS or cystic fibrosis or monetize the compound:** Following the positive MAD (multiple ascending dose) trial results reported in March 2021, Santhera is preparing for a phase IIa proof-of-concept (POC) trial of lonodelestat in ARDS or cystic fibrosis. Sufficient funding must be secured before starting the POC trial. Alternatively, the compound could be monetized. The program is currently paused, primarily focusing on successfully commercializing vamorolone in DMD.

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

**The Catalyst agreement provides a cash runway into Q1 2025 with funds to build an EU sales infrastructure for vamorolone and repay the short-term Highbridge facility**

The near-term proceeds from the agreement with Catalyst upon US approval of vamorolone in DMD amount to USD 100 mn (cash inflows of USD 126 mn net of regulatory approval milestone obligation to third parties of USD 26 mn). This substantial infusion of funds significantly extends Santhera's cash reach into Q1 2025. This provides a strong financial foundation for future activities, including advancing the development of vamorolone and European commercialization. In addition, funds will also be allocated towards the repayment of the exchangeable notes facility to Highbridge, resulting in a substantial reduction in debt and strengthening the company's financial position.

# Valuation Overview

**Sum-of-parts risk-adjusted (r)NPV points to a fair value of CHF 11.2 per share**
We derive a sum-of-parts rNPV of CHF 11.2 per share for Santhera, with estimated cash and cash equivalents (31 July 2023) of CHF 0.6 per share and overhead expenses of CHF 1.7 per share (including the repayment of convertible bonds of CHF 30.3 mn in 2024) assuming a WACC of 7% (reflecting the low Swiss interest environment).

**SUM OF PARTS**

| PRODUCT | INDICATION | PEAK SALES (CHF MN) | LAUNCH YEAR (EST) | UNADJUSTED NPV/SHARE (CHF) | SUCCESS PROBABILITY | RNPV/SHARE (CHF) | PERCENTAGE OF TOTAL |
|---|---|---|---|---|---|---|---|
| VAMOROLONE | DMD * (STEROID REPLACEMENT) | 709 | LATE 2023 | 14.5 | 80% | **11.6** | 90% |
| RAXONE | LHON ** - NON-CORE | 47 | 2015 | 0.6 | 100% | **0.6** | 5% |
| LONODELESTAT (PAUSED) | RARE ACUTE LUNG DISEASES | TBD | TBD | | | | |
| ESTIMATED CASH & CASH EQUIVALENTS (31 JULY 2023) | | 48 | | 0.6 | | **0.6** | 5% |
| **TOTAL ASSETS** | | | | 25.9 | | **12.9** | **100%** |
| OVERHEAD EXPENSES (INCL. REPAYMENT OF CONVERTIBLE BONDS DUE 2024) | | | | -1.7 | | **-1.7** | |
| **NPV/SHARE (CHF)** | | | | 24 | | **11.2** | |
| SHARE PRICE ON JUNE 26, 2023 | | | | | | 0.8 | |
| PERCENTAGE UPSIDE / (DOWNSIDE) | | | | | | 1318% | |

* DMD = DUCHENNE MUSCULAR DYSTROPHY; ** LHON = LEBER'S HEREDITARY OPTIC NEUROPATHY
ESTIMATES AS OF 26 JUNE 2023

SOURCE: VALUATIONLAB ESTIMATES

## Santhera's key drivers include:

**Vamorolone in DMD (all patients) – rNPV of CHF 11.6/share**
Vamorolone targets DMD patients with early-to-mid-stage disease to preserve muscle function, effectively replacing mainstay steroid therapy irrespective of the underlying genetic mutation. EU filing was completed in September 2022, followed by the US in October 2022 based on the positive pivotal phase IIb "VISION-DMD" trial. The FDA has set a 26 October 2023 Prescription Drug User Fee Act (PDUFA) date when it expects to complete its review. US launch by its North American commercialization partner Catalyst is expected in late Q4 2023. A positive CHMP recommendation in the EU is expected in late Q3 2023, with the first launch in Germany in late 2023, followed by the other EU member states in 2024. We forecast global peak sales of approximately CHF 700 mn for vamorolone in DMD alone. Additional sales in the near-term could come from Becker's muscular dystrophy (BMD), where ReveraGen received a USD 1.2 mn grant from the FDA and started a phase II pilot trial of vamorolone in adults and children with BMD with results due by the end of 2023. This grant further underlines the potential of vamorolone in other (rare) muscular disorders. In the US, we account for the short-term USD 126 mn cash inflow on the closing of the transaction (July 2023) and US approval of vamorolone in DMD, with tiered up to low-teen percentage sales royalties and Catalyst paying the royalty obligations (single-digit to low double-digit percentage royalties) to Idorsia and ReveraGen. In Europe, we account for the costs to build up a specialist sales force in the EU Top 5, Austria and Benelux countries with COGS of 10% and tiered single to double-digit royalty payments on sales to Idorsia and ReveraGen. In the Greater China Region, Sperogenix will commercialize vamorolone in return for up to USD 124 mn milestones (including additional indications) and double-digit royalties on net sales. In the ROW, we expect Santhera to seek partners in return for milestones and royalties on net sales. We calculate an rNPV of CHF 11.6/share for vamorolone in DMD alone, applying a historical 80% (filed) success rate.

**Raxone in LHON – rNPV of CHF 0.6/share**

In Europe, we conservatively expect Santhera to receive a total of CHF 30 mn in additional sales milestones (Santhera is eligible up to a maximum of EUR 49 mn) from Chiesi next to Raxone sales in LHON from France until the transition with Chiesi is completed. This amounts to an NPV of CHF 0.6 per share for the remaining Raxone revenues in LHON. We conservatively no longer include any revenues for Raxone in LHON in North America, where Santhera still owns the rights.

## Currently, no value attributed to early-stage pipeline projects

We have conservatively not accounted for Santhera's early-stage pipeline projects due to the lack of sufficient proof-of-concept at the moment.

**Lonodelestat in rare lung disorders– Phase II-ready - PAUSED**

In February 2018, Santhera acquired the exclusive global rights for lonodelestat, a novel, selective human neutrophil elastase (hNE) inhibitor for treating rare lung diseases from Polyphor (now Spexis) for CHF 6.5 mn in Santhera shares in a back-loaded agreement. In March 2021, Santhera reported positive topline results of a phase Ib MAD (multiple ascending dose) trial in cystic fibrosis patients. The trial was designed by Spexis and financially supported by the Cystic Fibrosis Foundation, underlining the high unmet medical need. Lonodelestat is phase II-ready for proof-of-concept (POC) in cystic fibrosis or acute respiratory distress syndrome (ARDS), both multibillion-dollar markets with few or no effective treatments. Additionally, imbalanced neutrophil activity is associated with many inflammatory diseases, which could be additional therapeutic indications for lonodelestat. For instance, lonodelestat could have potential in lung disorders associated with high hNE activity, including AAT (alpha-1 antitrypsin deficiency), NCFB (non-cystic fibrosis bronchiectasis), and PCD (primary ciliary dyskinesia).

Clinical development of lonodelestat has been paused due to cash restraints. Vamorolone is the main strategic focus and will consume all financial and human resources in the near term. Santhera explores various opportunities via collaboration and/or partnerships to resume the project as quickly as possible.

## Sensitivities that can influence our valuation

**Funding risk:** The Catalyst agreement, with a value of up to USD 231 mn with significant upfront payments, is guided to provide a cash runway into Q1 2025.

Santhera continues to evaluate the monetization of pipeline assets such as vamorolone in DMD in certain geographies outside the US and EU and in large non-DMD indications such as asthma, COPD, and ulcerative colitis.

**Development risk:** Vamorolone is Santhera's most advanced pipeline compound, awaiting US, EU, and UK approval in DMD in Q4 2023, with launches planned shortly after. Vamorolone has successfully completed pivotal phase III development with positive single pivotal phase IIb "VISION-DMD" trial results, backed by encouraging long-term POC trial data, which largely de-risks this novel DMD compound. We have not included forecasts for lonodelestat, which is phase II-ready for cystic fibrosis or ARDS until sufficient funding has been secured and the company announces in which indication it plans to start a POC trial.

**Approval risk:** Santhera focuses on developing drugs for orphan indications where regulatory requirements are typically less rigorous than for regular indications. For instance, conditional (EU) or accelerated (US) early approval can be granted without robust clinical trial data, e.g., based on a small, single clinical trial. However, sometimes the absence of a clear regulatory pathway on how to set up the pivotal clinical trial, including what the right endpoints should be, may add to the risk. We assume a historical 80% (filing) success rate for an orphan drug that has successfully completed phase III development.

**Pricing and reimbursement risk:** Following approval of vamorolone, pricing, and reimbursement have to be negotiated. In the US, pricing is quite straightforward, while in the EU, this has to be negotiated in each member state individually. Pricing could be lower, and negotiations could take longer than forecast. Orphan drugs such as vamorolone 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 vamorolone in DMD. In the EU Top 5, Austria, and Benelux countries Santhera plans to build up a small specialist sales force to commercialize vamorolone and maximize long-term profitability. 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 vamorolone. Santhera will be dependent on the sales efforts and marketing muscle of its partners 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 |
|---|---|---|---|---|---|
| **2023** | | | | | |
| | VAMOROLONE | NON-DMD* INDICATIONS & DMD OUTSIDE US/EUROPE | PARTNERING OPPORTUNITIES | PARTNERING VAMOROLONE NON-DMD INDICATIONS OR FOR DMD OUTSIDE NORTH AMERICA AND EUROPE FOR UPFRONT, REGULATORY AND SALES MILESTONES AND ROYALTIES ON SALES; POTENTIAL TO PROVIDE SIGNIFICANT NON-DILUTIVE FUNDING | |
| 9 JAN | VAMOROLONE | DMD | FDA ACCEPTANCE OF FILING | FDA ACCEPTED THE NDA FILING OF VAMOROLONE IN DMD AND SET A 26 OCTOBER 2023 PDUFA DATE; CURRENTLY THE FDA DOES NOT PLAN AN ADVISORY MEETING FOR VAMOROLONE IN DMD | |
| 10 JAN | | | IDORSIA SHARE AGREEMENT | THROUGH A SHARE EXCHANGE SANTHERA RECEIVED THE EQUIVALENT OF CHF 5 MN IDORSIA SHARES (346,500), WHICH SANTHERA IS FREE TO SELL TO SUPPORT ITS SHORT-TERM FINANANCIAL NEEDS, WITH IDORSIA RECEIVING 5,529,016 SANTHERA SHARES WITH THE IDORSIA STAKE IN SANTHERA INCREASING TO 17.7% | |
| 8 FEB | RAXONE | LHON** | AGREEMENT FRANCE | FINAL REIMBURSEMENT AGREEMENT WITH THE FRENCH AUTHORITIES RELATED TO RAXONE; NEWLY AGREED PRICE IS LOWER THAN PRICE UNDER THE TEMPORARY PRICING SCHEME, SANTHERA WILL MAKE REPAYMENTS OF AROUND CHF 25 MN (30% AROUND MID-2024, THE REMAINDER OF AROUND CHF 17.5 MN ONE YEAR LATER); SANTHERA PLANS TO SUBMIT VAMOROLONE FOR EARLY ACCESS IN FRANCE WITH FIRST SALES LIKELY IN H2 2024 AHEAD OF A POTENTIAL EMA APPROVAL | |
| | | | VAMORLONE EARLY ACCESS FILING FRANCE | | |
| 28 FEB | | | FUNDING UPDATE | FUNDING SECURED UNTIL OCTOBER 2023 THROUGH A PRIVATE PLACEMENT AND UPSIZING OF THE HIGHBRIDGE AGREEMENT; A STRATEGY COMMITTEE OF BOARD AND MANAGEMENT WAS FORMED; INCREASE OF SHARE CAPITAL FOR PRIVATE PLACEMENT AND FUTURE FINANCINGS | |
| 2 MAR | VAMOROLONE | DMD | UK FILING FOR APPROVAL<br>UK EAMS FILING | THE THIRD FILING FOR VAMOROLONE APPROVAL IN DMD WAS FILED IN THE UK WITH A POTENTIAL APPROVAL IN LATE 2023; SANTHERA IS ALSO PREPARING AN APPLICATION TO INCLUDE VAMOROLONE IN THE EAMS (EARLY ACCESS TO MEDICINES SCHEME) IN THE UK | |
| 25 APR | VAMOROLONE | DMD | MID-CYCLE FDA REVIEW MEETING | THE MID-CYCLE REVIEW MEETING BY THE FDA OF THE NDA (NEW DRUG APPLICATION) FOR VAMOROLONE IN DMD WAS SUCCESSFULLY COMPLETED; NO SIGNIFICANT REVIEW OR SAFETY CONCERNS WERE NOTED; THE FDA REAFFIRMED ITS DECISION TO FORGO AND ADVISORY COMMITTEE MEETING AND CONFIRMED THE 26 OCTOBER 2023 PDUFA TARGET DATE BY WHEN IT EXPECTS TO COMPLETE THE REVIEW | |
| 27 APR | | | PRELIMINARY 2022 RESULTS | PRELIMINARY UNAUDITED 2022 RESULTS: REVENUE FROM CONTRACTS WITH CUSTOMERS CHF 7.5 MN (2021: CHF -1.6 MN) BOOSTED BY SPEROGENIX MILESTONE, OPERATING RESULT CHF -52.0 MN (2021: CHF -56.9 MN), NET RESULT CHF -70.1 (2021: -55.5 MN) AFFECTED BY LARGER NET FINANCIAL EXPENSES OF CHF -17.7 MN (2021: 2.2 MN); CASH AND CASH EQUIVALENTS CHF 1.4 MN (31 DECEMBER 2022) TOGETHER WITH EXISTING FINANCING FACILITIES (HIGHBRIDGE) RESULTS IN A CASH REACH INTO Q4 2023 | |
| 31 MAY | | | FY 2022 RESULTS | FY 2022 AUDITED RESULTS LARGELY REFLECT THE PRELIMINARY UNAUDITED 2022 RESULTS REPORTED ON APRIL 27TH | |
| 20 JUN | VAMOROLONE | DMD | CATALYST LICENSES NORTH AMERICA RIGHTS | CATALYST PHARMACEUTICALS GAINED THE EXCLUSIVE LICENSE FOR NORTH AMERICA (US, CANADA, MEXICO) FOR VAMOROLONE IN DMD AND OTHER INDICATION IN A DEAL VALUED UP TO USD 231 MN; ON CLOSING (EARLY Q3) SANTHERA RECEIVES A USD 75 MN CASH UPFRONT AND USD 15 MN EQUITY INVESTMENT; ON US APPROVAL AN ADDITIONAL USD 10 MN REGULATORY MILESTONE PLUS USD 26 MN TO PAY APPROVAL-RELATED REGULATORY MILESTONES TO THIRD PARTIES (IDORSIA/REVERAGEN) AND POTENTIAL MILESTONES OF UP TO USD 105 MN; CATALYST WILL PAY LOW-TEEN ROYALTIES AND CORRESPONDING THIRD-PARTY ROYALTY OBLIGATIONS (ESTIMATED 5-7% ROYALTIES TO REVERAGEN) ON VAMOROLONE SALES IN NORTH AMERICA; CASH RUNWAY EXTENDED INTO Q1 2025 (FROM Q4 2023) | |
| 27 JUN | | | AGM | ANNUAL GENERAL MEETING (AGM) - INCLUDING A BOARD PROPOSAL FOR A REVERSE SHARE SPLIT IN THE RATIO OF 10 EXISTING SHARES FOR 1 NEW SHARE | |
| 26 OCT | VAMOROLONE | DMD | US APPROVAL | US PDUFA DATE WHEN THE FDA IS EXPECTED TO COMPLETE ITS REVIEW OF VAMOROLONE IN DMD | +CHF 1.4 |
| EARLY Q3 | VAMOROLONE | DMD | CLOSING CATALYST AGREEMENT | CLOSING OF CATALYST TRANSACTION, SUBJECT TO CUSTOMARY CONDITIONS AND REGULATORY CLEARANCES IN THE US | |
| Q3 | VAMOROLONE | DMD | CHMP OPINION | CHMP OPINION BASED ON 12 MONTHS TREATMENT DATA OF THE PIVOTAL PHASE IIB "VISION-DMD" TRIAL | +CHF 1.4 |
| LATE Q4 | VAMOROLONE | DMD | US LAUNCH | US LAUNCH OF VAMOROLONE TO TREAT DMD BY NORTH AMERICA PARTNER CATALYST PHARMACEUTICALS' RARE DISEASE SPECIALIST SALES FORCE | |
| LATE Q4 / EARLY Q1 2024 | VAMOROLONE | DMD | EU APPROVAL<br>INITIAL EU LAUNCHES | EU APPROVAL (MARKETING AUTHORIZATION APPLICATION) FOR DMD PATIENTS IS EXPECTED 67 DAYS AFTER POSITIVE CHMP OPINION; GERMANY EXPECTED TO BE FIRST EU MEMBER STATE TO LAUNCH IN LATE 2023 | |

* DMD = DUCHENNE MUSCULAR DYSTROPHY

ESTIMATES AS OF 26 JUNE 2023

SOURCE: VALUATIONLAB ESTIMATES

# Income Statement

| SANTHERA PHARMACEUTICALS | | | | | | | | | SHARE PRICE (CHF) | 0.79 |
|---|---|---|---|---|---|---|---|---|---|---|
| IFRS | | | | | | | | | | |
| **INCOME STATEMENT (CHF MN)** | **2022** | **2023E** | **2024E** | **2025E** | **2026E** | **2027E** | **2028E** | **2029E** | **2030E** | **2031E** | **2032E** |
| **PRODUCT SALES (INCL. PARTNER SALES)** | **46** | **46** | **121** | **189** | **340** | **441** | **535** | **677** | **855** | **1,013** | **1,173** |
| CHANGE (%) | 14% | 0% | 165% | 56% | 80% | 30% | 21% | 27% | 26% | 19% | 16% |
| **REPORTED SALES (SANTHERA TERRITORIES)** | **-6** | **3** | **23** | **62** | **100** | **145** | **169** | **259** | **395** | **522** | **670** |
| CHANGE (%) | 12% | -154% | 672% | 169% | 60% | 46% | 16% | 53% | 53% | 32% | 28% |
| **ROYALTIES (FROM PARTNER SALES)** | **2** | **0** | **4** | **8** | **20** | **33** | **43** | **53** | **59** | **63** | **64** |
| **UPFRONT & MILESTONES** | **11** | **107** | **34** | **42** | **18** | **9** | **27** | **4** | **2** | **47** | **0** |
| **OTHER REVENUES** | **0** | **0** | **0** | **0** | **0** | **0** | **0** | **0** | **0** | **0** | **0** |
| CHANGE (%) | 0% | 0% | 0% | 0% | 0% | 0% | 0% | 0% | 0% | 0% | 0% |
| **REVENUES (EXCL. PARTNER SALES)** | **7** | **110** | **62** | **112** | **138** | **187** | **238** | **316** | **455** | **632** | **734** |
| CHANGE (%) | -569% | 1376% | -44% | 82% | 22% | 36% | 28% | 33% | 44% | 39% | 16% |
| **COGS (INCL. PAYMENTS TO SPEXIS / IDORSIA)** | **-4** | **0** | **-3** | **-10** | **-11** | **-22** | **-26** | **-64** | **-58** | **-72** | **-91** |
| **GROSS PROFIT** | **4** | **110** | **59** | **103** | **127** | **164** | **212** | **252** | **398** | **560** | **643** |
| CHANGE (%) | -172% | 2738% | -47% | 75% | 24% | 30% | 29% | 19% | 58% | 41% | 15% |
| MARGIN (%) | 51.9% | 99.9% | 95.3% | 91.3% | 92.2% | 88.0% | 89.1% | 79.8% | 87.3% | 88.6% | 87.6% |
| **R&D** | **-31** | **-28** | **-22** | **-39** | **-20** | **-23** | **-27** | **-9** | **-10** | **-11** | **-12** |
| CHANGE (%) | 3% | -8% | -22% | 78% | -48% | 12% | 18% | -67% | 11% | 10% | 9% |
| **GENERAL & ADMINISTRATIVE** | **-15** | **-15** | **-16** | **-16** | **-17** | **-18** | **-19** | **-20** | **-21** | **-22** | **-23** |
| CHANGE (%) | 14% | 2% | 5% | 5% | 5% | 5% | 5% | 5% | 5% | 5% | 5% |
| **MARKETING & SALES** | **-11** | **-14** | **-11** | **-13** | **-13** | **-17** | **-20** | **-74** | **-103** | **-122** | **-146** |
| CHANGE (%) | 16% | 32% | -24% | 20% | 3% | 29% | 17% | 265% | 39% | 19% | 19% |
| **OTHER OPERATING INCOME/(EXPENSE)** | **0** | **0** | **0** | **0** | **0** | **0** | **0** | **0** | **0** | **0** | **0** |
| **EBIT** | **-52** | **53** | **11** | **34** | **76** | **106** | **146** | **149** | **264** | **405** | **462** |
| CHANGE (%) | -9% | -202% | -80% | 227% | 121% | 40% | 37% | 2% | 77% | 54% | 14% |
| MARGIN (%) | -696% | 48% | 17% | 31% | 55% | 57% | 61% | 47% | 58% | 64% | 63% |
| **EBITDA** | **-42** | **55** | **13** | **37** | **78** | **108** | **148** | **152** | **266** | **408** | **465** |
| CHANGE (%) | -21% | -231% | -77% | 190% | 114% | 39% | 37% | 2% | 76% | 53% | 14% |
| MARGIN (%) | -564% | 50% | 20% | 32% | 57% | 58% | 62% | 48% | 58% | 64% | 63% |
| **D&A** | **10** | **2** | **2** | **2** | **2** | **2** | **2** | **2** | **2** | **2** | **2** |
| **NET FINANCIAL INCOME/(EXPENSES)** | **-19** | **-4** | **-6** | **6** | **6** | **7** | **7** | **7** | **8** | **8** | **9** |
| **PROFIT/(LOSS) BEFORE TAXES** | **-71** | **49** | **5** | **40** | **82** | **113** | **153** | **157** | **272** | **413** | **471** |
| CHANGE (%) | 29% | -170% | -90% | 726% | 104% | 37% | 36% | 2% | 73% | 52% | 14% |
| MARGIN (%) | -945% | 45% | 8% | 36% | 60% | 60% | 64% | 50% | 60% | 65% | 64% |
| **TAXES (EXCL. TAX LOSS CARRYFORWARDS)** | **0** | **0** | **0** | **0** | **-5** | **-22** | **-38** | **-37** | **-39** | **-50** | **-42** |
| TAX RATE (%) | -1% | 0% | 0% | 0% | 6% | 19% | 25% | 24% | 14% | 12% | 9% |
| **NET PROFIT/LOSS** | **-71** | **49** | **5** | **40** | **77** | **91** | **115** | **119** | **232** | **363** | **429** |
| CHANGE (%) | 28% | -169% | -90% | 726% | 91% | 18% | 26% | 4% | 95% | 56% | 18% |
| MARGIN (%) | -951% | 45% | 8% | 36% | 56% | 49% | 48% | 38% | 51% | 57% | 58% |
| **EPS (CHF)** | **-1.17** | **0.65** | **0.06** | **0.54** | **1.03** | **1.21** | **1.53** | **1.59** | **3.08** | **4.82** | **5.70** |

ESTIMATES AS OF 26 JUNE 2023 SOURCE: VALUATIONLAB ESTIMATES

**NOTE:** On 31 December 2022, Santhera had a total of CHF 290.8 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) | 0.79 |
|---|---|---|---|---|---|---|---|---|---|---|
| **IFRS** | | | | | | | | | | |
| **RATIOS** | **2022** | **2023E** | **2024E** | **2025E** | **2026E** | **2027E** | **2028E** | **2029E** | **2030E** | **2031E** | **2032E** |
| P/E | | 1.2x | 12.2x | 1.5x | 0.8x | 0.7x | 0.5x | 0.5x | 0.3x | 0.2x | 0.1x |
| P/S | | 0.5x | 1.0x | 0.5x | 0.4x | 0.3x | 0.3x | 0.2x | 0.1x | 0.1x | 0.1x |
| P/NAV | | -4.8x | -1.8x | 8.4x | 0.7x | 0.3x | 0.2x | 0.1x | 0.1x | 0.1x | 0.0x |
| EV/EBITDA | | 0.8x | 3.3x | 1.1x | 0.5x | 0.4x | 0.3x | 0.3x | 0.2x | 0.1x | 0.1x |
| **PER SHARE DATA (CHF)** | **2022** | **2023E** | **2024E** | **2025E** | **2026E** | **2027E** | **2028E** | **2029E** | **2030E** | **2031E** | **2032E** |
| **EARNINGS** | **-1.17** | **0.65** | **0.06** | **0.54** | **1.03** | **1.21** | **1.53** | **1.59** | **3.08** | **4.82** | **5.70** |
| CHANGE (%) | -28% | -156% | -90% | 726% | 91% | 18% | 26% | 4% | 95% | 56% | 18% |
| **CASH** | **0.02** | **0.46** | **0.21** | **0.77** | **1.89** | **3.42** | **5.49** | **7.60** | **11.23** | **16.75** | **23.04** |
| CHANGE (%) | -96% | 1963% | -54% | 268% | 145% | 81% | 60% | 38% | 48% | 49% | 38% |
| **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** | **-0.72** | **-0.17** | **-0.44** | **0.09** | **1.12** | **2.33** | **3.86** | **5.44** | **8.53** | **13.35** | **19.05** |
| CHANGE (%) | -1945% | -77% | 165% | -121% | 1085% | 108% | 66% | 41% | 57% | 57% | 43% |
| **BALANCE SHEET (CHF MN)** | **2022** | **2023E** | **2024E** | **2025E** | **2026E** | **2027E** | **2028E** | **2029E** | **2030E** | **2031E** | **2032E** |
| **NET LIQUID FUNDS** | **1** | **34** | **16** | **58** | **143** | **258** | **413** | **572** | **846** | **1,262** | **1,735** |
| **TOTAL ASSETS** | **64** | **97** | **78** | **121** | **205** | **320** | **476** | **635** | **908** | **1,324** | **1,798** |
| **TOTAL SHAREHOLDERS' EQUITY** | **-44** | **-13** | **-33** | **7** | **84** | **175** | **290** | **410** | **642** | **1,005** | **1,435** |
| CHANGE (%) | | | | | | | | | | | |
| RETURN ON EQUITY (%) | 163% | -392% | -15% | 567% | 92% | 52% | 40% | 29% | 36% | 36% | 30% |
| **FINANCIAL DEBT** | **51** | **51** | **21** | **0** | **0** | **0** | **0** | **0** | **0** | **0** | **0** |
| **EMPLOYEES** | 51 | 52 | 55 | 55 | 56 | 56 | 57 | 57 | 58 | 59 | 59 |
| *- CHANGE IN %* | *19%* | *2%* | *5%* | *1%* | *1%* | *1%* | *1%* | *1%* | *1%* | *1%* | *1%* |
| **CASH FLOW STATEMENT (CHF MN)** | **2022** | **2023E** | **2024E** | **2025E** | **2026E** | **2027E** | **2028E** | **2029E** | **2030E** | **2031E** | **2032E** |
| **PROFIT / (LOSS) BEFORE TAXES** | **-71** | **49** | **5** | **40** | **82** | **113** | **153** | **157** | **272** | **413** | **471** |
| DEPRECIATION & AMORTIZATION | 10 | 2 | 2 | 2 | 2 | 2 | 2 | 2 | 2 | 2 | 2 |
| OTHER NON-CASH ITEMS | 31 | | | | | | | | | | |
| **CASH FLOW FROM OPERATING ACTIVITIES** | **-30** | **51** | **7** | **42** | **84** | **115** | **155** | **159** | **274** | **416** | **473** |
| CASH FLOW FROM INVESTING ACTIVITIES | -4 | | | | | | | | | | |
| **FREE CASH FLOW** | **-34** | **51** | **7** | **42** | **84** | **115** | **155** | **159** | **274** | **416** | **473** |
| CASH FROM FINANCING ACTIVITIES | 14 | -18 | -26 | 0 | 0 | 0 | 0 | 0 | 0 | 0 | 0 |
| EFFECTS OF EXCHANGE RATE CHANGES ON CASH | 0 | | | | | | | | | | |
| **CHANGE IN LIQUID FUNDS** | **-20** | **33** | **-19** | **42** | **84** | **115** | **155** | **159** | **274** | **416** | **473** |

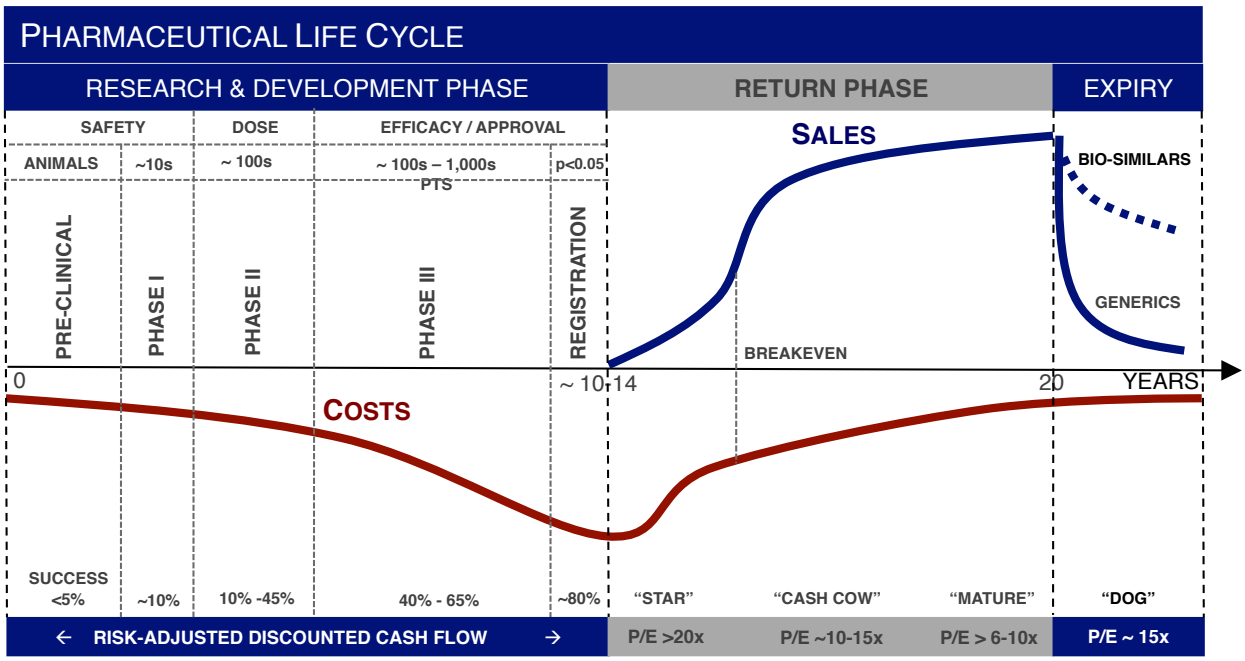
ESTIMATES AS OF 26 JUNE 2023 SOURCE: VALUATIONLAB ESTIMATES

**NOTE:** Following the recent exclusive license agreement with Catalyst for vamorolone in North America (US, Canada, Mexico) with a substantial influx of cash guides for a cash runway into Q1 2025 (from Q4 2023).

# APPENDIX

## 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

valuationLAB AG is an independent life science research boutique with no securities or banking services. The company does not hold any positions in the securities mentioned in this report.

**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) |

## Analyst Certification

The research analyst(s) identified on the first page of this research report hereby attest that all of the views expressed in this report accurately reflect their personal views about any and all of the subject securities or issuers. In order to ensure the independence of our research analysts, and their immediate household, are expressly prohibited from owning any securities in the valuationLAB AG research universe, which belong to their sector(s). Neither the research analyst nor his/her immediate household serves as an Officer, Director or Advisory Board Member of Santhera Pharmaceuticals Holding AG.