Santhera Pharmaceuticals

Developing medicines to meet the needs of patients living with rare diseases

Corporate Presentation

June 2023
Disclaimer

This presentation is not and under no circumstances to be construed as a solicitation, offer, or recommendation, to buy or sell securities issued by Santhera Pharmaceuticals Holding AG. Santhera Pharmaceuticals Holding AG makes no representation (either express or implied) that the information and opinions expressed in this presentation are accurate, complete or up to date. Santhera Pharmaceuticals Holding AG disclaims, without limitation, all liability for any loss or damage of any kind, including any direct, indirect or consequential damages, which might be incurred in connection with the information contained in this presentation.

This presentation expressly or implicitly contains certain forward-looking statements concerning Santhera Pharmaceuticals Holding AG and its business. Certain of these forward-looking statements can be identified by the use of forward-looking terminology or by discussions of strategy, plans or intentions. Such statements involve certain known and unknown risks, uncertainties and other factors, which could cause the actual results, financial condition, performance or achievements of Santhera Pharmaceuticals Holding AG to be materially different from any expected results, performance or achievements expressed or implied by such forward-looking statements. There can be no guarantee that any of the research and/or development projects described will succeed or that any new products or indications will be brought to market. Similarly, there can be no guarantee that Santhera Pharmaceuticals Holding AG or any future product or indication will achieve any particular level of revenue. In particular, management’s expectations could be affected by, among other things, uncertainties involved in the development of new pharmaceutical products, including unexpected preclinical and clinical trial results; unexpected regulatory actions or delays or government regulation generally; the Santhera Pharmaceuticals Holding AG’s ability to obtain or maintain patent or other proprietary intellectual property protection; competition in general; government, industry, and general public pricing and other political pressures. Santhera Pharmaceuticals Holding AG is providing the information in this presentation as of the date of the publication, and does not undertake any obligation to update any forward-looking statements contained herein as a result of new information, future events or otherwise.
1. **SIX Swiss Exchange listed company (SANN)**
   - Global headquarters near Basel (Switzerland) with internationally experienced leadership team
   - North American headquarters near Boston (USA)

2. **Vamorolone in Duchenne muscular dystrophy close to regulatory decisions**
   - Filings under review in US, EU and UK with decisions expected in Q4-2023
   - Vamorolone as foundational therapy and alternative to standard of care corticosteroids
   - Differentiated safety profile addresses needs across broad patient segments
   - Potential as alternative to corticosteroids in broad range of therapeutic indications

3. **Neutrophil elastase inhibitor Ionoidelestat Phase 2 ready in pulmonary indications**
   - Successful multiple ascending dose study in cystic fibrosis patients completed
   - Novel anti-inflammatory agent for neutrophil associated pulmonary disorders in general

4. **Finance**
   - Licensing of vamorolone in North America with deal value up to USD 231 million on June 19, 2023
   - Cash runway into Q1-2025
   - Major shareholders*: Catalyst Pharmaceuticals, Inc. 11.3%; Idorsia 10.4%

* Pro forma statement after closing of transaction expected early Q3-2023
Santhera pipeline with two assets and broad therapeutic potential
Opportunities beyond current active program in Duchenne muscular dystrophy (DMD)

Vamorolone foundational therapy in DMD
- US NDA, EU MAA, and UK MAA under review for potential approval in Q4-2023
- Positive pivotal data in Phase 2b as well as long-term extension study
- Potential as alternative to corticosteroids in broad range of therapeutic areas
- Own commercialization in top-5 Europe (Germany, UK, France, Italy, Spain), plus Benelux, Austria. Commercialization in other countries via partner(s)
- Peak potential > EUR 150 million in DMD (in top-5 EU markets)¹
- Commercialization in the US by partner Catalyst, in China by partner Sperogenix

Lonodelestat targeting inflammation pulmonary disease
- Positive MAD Phase 1b trial in cystic fibrosis
- Safe dose regimen; effect on biomarker
- Potential in inflammatory lung diseases with neutrophil involvement, both for acute & chronic application
- Program Phase 2 ready in CF and ARDS, development currently paused by Santhera due to funding limitations
- Open for development partnerships

Worldwide rights for all indications for both assets (vamorolone partnered in North America & China)

¹: Santhera estimate; MAD: Multiple Ascending Dose; CF: Cystic Fibrosis; ARDS: Acute Respiratory Distress Syndrome
# Lead asset vamorolone in DMD close to regulatory decisions

US NDA, EU & UK MAA, under review for potential approval in Q4-2023 (US FDA PDUFA date Oct 26, 2023)

<table>
<thead>
<tr>
<th>Molecule</th>
<th>Indication</th>
<th>IND</th>
<th>Ph 1</th>
<th>PoC</th>
<th>Pivotal</th>
<th>Filing</th>
<th>Market</th>
<th>Milestones and remarks</th>
</tr>
</thead>
</table>
| **Vamorolone¹** | • dissociative steroid  
• oral suspension  
Duchenne muscular dystrophy | | | | | | | North America & China partnerships |
| | Becker muscular dystrophy | | | | | | Trial under FDA grant to partner |
| | Steroid alternative in multiple pediatric rare indications | | | | | | Under evaluation |
| **Lonodelestat²** | • hNE inhibitor  
• via nebulizer  
Cystic fibrosis | | | | | | Phase 2 ready for CF and ARDS (currently paused) |
| | Multiple respiratory conditions with high hNE activity | | | | | | Under evaluation |

1: Vamorolone: Worldwide license from ReveraGen in Sep 2020  
License to Sperogenix for China in Jan 2022  
License to Catalyst for North America in Jun 2023; 2: Lonodelestat: Worldwide license from Spexis in Feb 2018  
hNE: Human Neutrophil Elastase; PoC: Proof of Concept; IND: Investigational new drug; NDA: New Drug Application;  
FDA: Food and Drug Administration; ARDS: Acute Respiratory Distress Syndrome; MAA: Marketing Authorization Application
Santhera Grants Exclusive North America License for Vamorolone to Catalyst Pharmaceuticals in Deal Valued at up to USD 231 Million Plus Royalties

- Santhera will receive USD 90 million upfront at closing (USD 75 million in cash and USD 15 million equity investment), an additional USD 10 million upon U.S. FDA approval of vamorolone in Duchenne muscular dystrophy (DMD) plus USD 26 million to pay approval related regulatory milestones to third parties, and potential sales milestones of up to USD 105 million
- Agreement covers commercialization of vamorolone in DMD and rights to all potential future indications in North America (NA)
- Catalyst will pay Santhera up to low-teem percentage royalties and will assume corresponding third-party royalty obligations on vamorolone sales in NA
- Santhera will continue to focus on European commercialization of vamorolone in DMD, and further development of its clinical pipeline
- Santhera and Catalyst intend to collaborate on joint clinical development and funding of vamorolone for additional indications beyond DMD
- Proceeds allow for repayment of all short-term debt with Highbridge and an overall strengthening of the balance sheet
Vamorolone in Duchenne muscular dystrophy and potentially other inflammatory disorders
DMD offers attractive opportunity in well-defined orphan disease market

**DMD market with few current treatment options, projected to be worth > USD 4 billion by 2023***
- Approx. 30,000 – 35,000 patients in US and Europe combined
- Well defined standard of care with corticosteroids as lead chronic treatment in established guidelines
- Patients diagnosed at early age and accessible
- Limited number of specialized centers
- Well knowledgeable patient advocacy groups
- Newer therapies likely to be used in combination with corticosteroids

**Current approved therapies command high price with intrinsic limitations to serve addressable market**
- Exon skippers and read through therapies serve niche segments based on genetic mutation
- Gene therapies deliver micro-dystrophin partially restoring function with re-dosing challenges
- Deflazacort (corticosteroid) is approved in US (Emflaza®), achieves attractive margins

**Focused expert centres treating patients in EU and US**

<table>
<thead>
<tr>
<th>DMD</th>
<th>Centers</th>
<th>HCPs</th>
</tr>
</thead>
<tbody>
<tr>
<td>US</td>
<td>~90</td>
<td>~450</td>
</tr>
<tr>
<td>EU4+UK</td>
<td>~180</td>
<td>~750</td>
</tr>
</tbody>
</table>

* Grand View Research Inc., Research & Markets, Decision Resources
The need for a better foundational steroid therapy in DMD

**EFFICACY OF STEROIDS**
Classical corticosteroids demonstrate efficacy with delay in disease progression.

They are used on top of exon skipping and read-through drugs or gene therapies under development

**ESTABLISHED FOUNDATIONAL THERAPY**

**SAFETY ISSUES WITH STEROIDS**
Classical corticosteroids are associated with significant side effect burden.

This leads to hesitance starting therapy in young boys, to underdosing and to early discontinuation.

**TOO LATE TOO LITTLE TOO SHORT**

**VAMOROLONE OFFERING**
Subtle difference in steroid ring structure leads to dissociative properties.

Maintained anti-inflammatory efficacy with improved safety profile has been established.

**NEW DISSOCIATIVE STEROID CLASS**

Differential profile covered in clinical section
Corticosteroids delay disease progression in DMD by 2 – 3 years\textsuperscript{4,6}

Established endpoints and consistent evidence base through several clinical studies

Corticosteroids are the standard of care

- DMD progression is sequential, non-linear and irreversible\textsuperscript{1-4}
- Early initiation of corticosteroids preserves muscle function and strength, delaying time to loss of functional milestones by 2 – 3 years\textsuperscript{4,6}
- Steroid treatment associated with a reduction in all-cause mortality, new onset and progressive cardiomyopathy\textsuperscript{5}

Corticosteroid treatment is associated with well-defined toxicities

...up to 65% of DMD patients discontinue treatment early due to adverse events\textsuperscript{1-3}

- 37% Growth failure/delay\textsuperscript{2,3}
- 37% Behavioral changes\textsuperscript{3}
- 30% Bone changes or fractures\textsuperscript{2,3}
- 55% Cushingoid appearance\textsuperscript{3}
- 65% Weight gain\textsuperscript{3}
- 31% Excessive hair growth\textsuperscript{2,3}
- 19% Cataracts\textsuperscript{2}

Corticosteroid use is limited due to known side effect profile

Use of corticosteroids in DMD is high, particularly in ambulatory patients, but declines with age\textsuperscript{1-4}

Reasons (%) for Discontinuing Steroid Treatment\textsuperscript{4}
- Problems with side effects
- Not enough benefits
- Did not like use of long-term medication
- Other

Reasons (%) for not Initiating Steroid Treatment\textsuperscript{4}
- Worried about side effects
- Doctor never prescribed/recommended
- Does not like use of long-term medication
- Other
- Worried about not getting enough benefit
- Age 3 and under

Diagnosed patients with DMD

\begin{itemize}
\item CS user
\item CS non-user
\end{itemize}
Vamorolone retains benefits of steroids with fewer side effects$^{1-3}$

**Like corticosteroids$^{4-5}$**
- Inhibition of NF-κB pro-inflammatory transcription factors

**Retained efficacy due to potent anti-inflammatory action**

**Unlike corticosteroids$^{4-5}$**
- Not a substrate of hydroxysteroid dehydrogenase
- Less activation of genes responsible for side effects
- Potent mineralocorticoid antagonist (eplerenone-like)
- Membrane stabilizer

Potential for significant reduction of steroid-associated side effects

---

Double bond in vamorolone chemical structure attenuates GC receptor binding and ultimately leads to less activation of genes responsible for side effects$^{4-5}$

Glucocorticoid Receptor Ligand Binding Domain

Vamorolone

Glucocorticoid Receptor Ligand Binding Domain

Vamorolone

Double bond in vamorolone chemical structure attenuates GC receptor binding and ultimately leads to less activation of genes responsible for side effects$^{4-5}$

---

**Comprehensive vamorolone development**

200 patient-years exposure in 160 DMD boys treated with vamorolone for up to 6 years¹

<table>
<thead>
<tr>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td>Study</td>
<td>Ph 1 (N=86)</td>
<td>Ph 2a (N=48)</td>
<td>Ph 2a Long-term Extension</td>
<td>Expanded Access Program (N=153)</td>
<td>Long term follow-up*</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

**Pivotal VISION-DMD (N=121) age 4 – <7 years**

**FOR-DMD External Comparator Study (N=196)**¹⁰

**Pivotal study** establishes efficacy vs placebo and comparable to prednisone (at week 24), maintenance of effect (at week 48) and safety differentiation

**External comparison** of pivotal study with matched patients from steroid use (prednisone and deflazacort) study strengthens safety differentiation at week 48

**2.5 year comparison** study with matched patients from steroid use (prednisone and deflazacort) study demonstrates safety differentiation in the long term

**Supportive Data**

- Study-006: supportive data in patients 2-18 years of age
- Study-008: effectiveness in 7-18 year old ambulatory / non-ambulatory switchers

---

Pivotal VISION-DMD: Study design
Randomized, double-blind, placebo and active control trial in 121 steroid-naive patients, aged 4 – <7 years

Outcome measures
Primary efficacy outcome measure: TTSTAND velocity vs placebo at 24 weeks
Key secondary outcome measures: 6MWT, TTRW, TTCLIMB, NSAA, safety and tolerability
Primary endpoint met with high statistical significance at 24 weeks
Consistent and robust efficacy shown by primary endpoint and majority of secondary endpoints for both vamorolone doses

<table>
<thead>
<tr>
<th>Rank</th>
<th>Endpoint</th>
<th>Comparison vs placebo</th>
<th>Difference</th>
<th>MCID</th>
<th>P-value</th>
</tr>
</thead>
<tbody>
<tr>
<td>Primary</td>
<td>TTSTAND velocity</td>
<td>vam 6mg/kg</td>
<td>0.06 rises/s</td>
<td>&gt;0.023 rises/s&lt;sup&gt;1&lt;/sup&gt;</td>
<td>0.002</td>
</tr>
<tr>
<td></td>
<td>TTSTAND velocity</td>
<td>vam 2mg/kg</td>
<td>0.04 rises/s</td>
<td>&gt;0.023 rises/s&lt;sup&gt;1&lt;/sup&gt;</td>
<td>0.017</td>
</tr>
<tr>
<td></td>
<td>6MWT</td>
<td>vam 6mg/kg</td>
<td>42 m</td>
<td>&gt;26-32 m&lt;sup&gt;2,3&lt;/sup&gt;</td>
<td>0.003</td>
</tr>
<tr>
<td></td>
<td>6MWT</td>
<td>vam 2mg/kg</td>
<td>37 m</td>
<td>&gt;26-32 m&lt;sup&gt;2,3&lt;/sup&gt;</td>
<td>0.009</td>
</tr>
<tr>
<td></td>
<td>TTRW velocity</td>
<td>vam 6mg/kg</td>
<td>0.24 m/s</td>
<td>&gt;0.2 m/s&lt;sup&gt;1,2&lt;/sup&gt;</td>
<td>0.002</td>
</tr>
<tr>
<td></td>
<td>TTRW velocity</td>
<td>vam 2mg/kg</td>
<td>0.13 m/s</td>
<td>&gt;0.2 m/s&lt;sup&gt;1,2&lt;/sup&gt;</td>
<td>0.103</td>
</tr>
<tr>
<td>Pre-Specified, Hierarchical Secondary</td>
<td>TTCLIMB velocity</td>
<td>vam 6mg/kg</td>
<td>0.07 task/s</td>
<td></td>
<td>&lt;0.001</td>
</tr>
<tr>
<td></td>
<td>TTCLIMB velocity</td>
<td>vam 2mg/kg</td>
<td>0.06 task/s</td>
<td></td>
<td>0.006</td>
</tr>
<tr>
<td></td>
<td>NSAA</td>
<td>vam 6mg/kg</td>
<td>3.4 points</td>
<td>&gt;2-3 points&lt;sup&gt;4,5&lt;/sup&gt;</td>
<td>&lt;0.001</td>
</tr>
<tr>
<td></td>
<td>NSAA</td>
<td>vam 2mg/kg</td>
<td>3.2 points</td>
<td>&gt;2-3 points&lt;sup&gt;4,5&lt;/sup&gt;</td>
<td>&lt;0.001</td>
</tr>
</tbody>
</table>

Primary endpoint met with clinically relevant treatment difference

Observed difference of 0.06 rises/sec is expected to delay the time to loss of ambulation by 2-3 years

1. McDonald et al. PPDM Conf. 2021 Poster #16, 2. mITT-1: modified intention to treat population from period 1, MMRM estimates of changes from baseline, 3. Press Release June 1, 2021, descriptive statistics
Comparable efficacy of vamorolone 6 mg/kg/d vs prednisone 0.75 mg/kg/d

Difference between groups in percentual change from baseline at week 24 (post hoc analysis)

Data on file (adapted from Poster 524 presented at WMS 2021), mITT-1

PDN: Prednisone 0.75 mg/kg/d; VAM: Vamorolone at 2 and 6 mg/kg/d; Time to Stand (TTSTAND), 6 Minute Walk Test (6MWT), Time to Run/Walk 10m (TTRW), Time to Climb 4 Stairs (TTCLIMB), North Star Ambulatory Assessment (NSAA).
No loss of efficacy when switching from prednisone to vamorolone

Durable treatment effect maintained over 48 weeks with vamorolone 6 mg/kg/d

- During treatment period 1, patients on vamorolone 6 mg/kg/d showed same change in TTSTAND velocity as patients on prednisone before switching to vamorolone 6 mg/kg/d
- During treatment period 2, both groups showed same maintenance of effect
- Historical data consistently show that there is no further improvement with prolonged steroid treatment after the initial improvement in TTSTAND

1. Data on File VAM-2021-002, mITT-2: modified intention to treat population from period 1 and 2, MMRM estimates of changes from baseline. PDN: prednisone 0.75mg/kg/day, PDN-VAM: prednisone 0.75 mg/kg/d in Period 1 transitioned to vamorolone 6mg/kg/d in Period 2 group after a 4-week tapering period; 2. McDonald et al. Poster PPMD Annual Conference 2021
The FOR-DMD study provides external comparator data

Pre-specified analyses in double-blind, randomized, academic-run, independent study

<table>
<thead>
<tr>
<th>Time point</th>
<th>Efficacy</th>
<th>Safety</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>Comparison</td>
<td>Method</td>
</tr>
<tr>
<td>24 weeks / 6 months</td>
<td>PDN (VISION-DMD) vs PDN (FOR-DMD)</td>
<td>Propensity score matching²</td>
</tr>
<tr>
<td>48 weeks / 12 months</td>
<td>VAM vs PDN vs DFZ</td>
<td>Propensity score matching²</td>
</tr>
<tr>
<td>2.5 years⁴</td>
<td>Not applicable</td>
<td>Not applicable</td>
</tr>
</tbody>
</table>

1. Guglieri et al JAMA 2022 doi:10.1001/jama.2022.4315, 2. Pre-defined propensity scores calculated based on baseline age, TTSTAND, NSAA score, height and weight; analysis weighted by the propensity scores. Patients meeting the common inclusion criteria of all studies are included. 3. For safety endpoints that require a long follow-up time, e.g., fractures. 4. Mah et al JAMA Network Open 2022 e2144178. doi:10.1001/jamaneurol2open.2021.44178. Efficacy and safety comparisons pre-specified.
VISION-DMD pre-specified* analyses vs FOR-DMD external control

Propensity matched cross study comparison shows comparable efficacy for vamorolone 6 mg/kg/d versus standard of care corticosteroid treatment

PDN: prednisone; VAM: vamorolone; DFZ: deflazacort

* Cross study comparisons with FOR-DMD as external control specified prior to data base lock in the statistical analysis plan of the VISION-DMD pivotal study.
Fewer and less severe spinal fractures with vamorolone compared to classical corticosteroids over 2.5 years

Vamorolone long-term extension (LTE) study vs FOR-DMD, matched comparison, central reading using modified Genant grades¹

Spinal Deformity Index (SDI): sum of the Genant Grades from T4 to L4, and therefore, is the composite of both fracture number and severity

Reduction of number of patients with vertebral fractures by approx. 50%
Bone biomarker data from VISION-DMD study supports findings on long-term bone health

Unlike classical corticosteroids, vamorolone does not have a negative impact

Biomarkers of bone formation

Osteocalcin (ng/ml)

P1NP (ng/ml)

CTX1 (pg/ml)

Biomarkers of bone remodelling

1. Data on File: VAM-2021-007, PDN, prednisone; SEM, standard error of mean; VAM, vamorolone. CTX1, C-terminal telopeptide of type 1 collagen; P1NP, procollagen type 1 N-terminal pro-peptide. Safety population (SAF-1) at 24 weeks, pre-specified analysis.
Bone biomarker data from VISION-DMD study supports findings on long-term bone health

Rapid recovery of bone biomarkers after switching from prednisone

Biomarkers of bone formation

- Osteocalcin (ng/ml)
- P1NP (ng/ml)

Biomarkers of bone remodelling

- CTX1 (pg/ml)

1. Data on File 2022, PDN, prednisone; VAM, vamorolone. CTX1, C-terminal telopeptide of type 1 collagen; P1NP, procollagen type 1 N-terminal pro-peptide. Safety population (SAF-2), change from baseline to week 48
Vamorolone allows for normal bone development and growth
Comparison to natural history data and in patients switching from prednisone

Vamorolone did not stunt growth unlike other corticosteroids used in DMD

Modelling of height trajectory from long-term vamorolone data and corticosteroids from CINRG Natural History Data²

Vamorolone did not stunt growth unlike other corticosteroids used in DMD

Switching from prednisone to vamorolone recovers normal growth trajectory (VISION-DMD study)

1. Safety Population 2 (SAF-2); PDN – Prednisone 0.75 mg/kg/d; PDN-VAM: growth trajectory (z-score) compared for prednisone in Period 1 and vamorolone (2 + 6 mg/kg/d) in Period 2; All doses daily; MMRM estimates of changes from baseline 2. Mah et al; ePoster LB.08 WMS 2021
Fewer and less severe behavioral problems reported with vamorolone

Comparison of behavioral problems reported for vamorolone vs prednisone at week 24

<table>
<thead>
<tr>
<th>VISION-DMD Study</th>
<th>Placebo N = 29</th>
<th>Prednisone 0.75 mg/kg/d N = 31</th>
<th>Vamorolone 2 mg/kg/d N = 30</th>
<th>Vamorolone 6 mg/kg/d N = 28</th>
</tr>
</thead>
<tbody>
<tr>
<td>Behavior problems AESIs, N (%)</td>
<td>4 (13.8)</td>
<td>10 (32.3)</td>
<td>5 (16.7)</td>
<td>6 (21.4)</td>
</tr>
<tr>
<td>Moderate/severe AESIs, N (%)</td>
<td>1 (3.4)</td>
<td>7 (22.6)</td>
<td>1 (3.3)</td>
<td>-</td>
</tr>
<tr>
<td>AESIs leading to discontinuation, N (%)</td>
<td>0</td>
<td>1 (3.2)</td>
<td>0</td>
<td>0</td>
</tr>
</tbody>
</table>

**PARS III subscales**

**PARS III scale:** proportion of patients shifting from normal to a clinically relevant worsening by subscale, defined as shift from normal adjustment score (z-score <1) at baseline to abnormal adjustment score (z-score ≥1) at Week 24 based on normative data from Henriksen 2009.
Vamorolone value proposition

• **Durable efficacy comparable to standard of care with vamorolone 6mg/kg/day**
  • Statistically robust efficacy vs placebo at 24 weeks for both 2mg/kg/day and 6mg/kg/day
  • No loss of efficacy when switching from prednisone to vamorolone
  • Long-term efficacy of vamorolone 6mg/kg/day comparable to standard of care glucocorticoid at 48 weeks

• **Preserved bone health with vamorolone, unlike deleterious effect of standard of care glucocorticoids (GC)**
  • Normal bone turnover biomarkers and reduction of risk of spinal fractures with long-term treatment vs GCs
  • Height trajectory as expected from CDC normalized growth curves unlike GCs and comparable to placebo

• **Improved safety profile compared to prednisone evident in the first 24 weeks**
  • Placebo-like Treatment Emergent Adverse Events (TEAEs) with vamorolone 2mg/kg/day
  • Fewer and milder TEAEs with vamorolone 6mg/kg/day compared to prednisone, including behavioral problems.

• **Effective 3-fold dose range with a dose-dependent safety profile allows for individualized dose adjustment as needed to best manage tolerability to maintain treatment long-term**
Regulatory decisions for vamorolone in DMD expected in Q4-2023

- Regulatory submissions are accepted and under review in the US, EU and UK for potential approval in Q4-2023
- Dossier is based on pivotal VISION-DMD study and long-term extension data compared to FOR-DMD natural history study
- Early access programs (EAP) submitted in the UK and France, once approved, could allow treatment of first patients in Q3-2023

### Regulatory Timeline

<table>
<thead>
<tr>
<th>2022</th>
<th>2023</th>
<th>2024</th>
</tr>
</thead>
<tbody>
<tr>
<td>Q3</td>
<td>Q4</td>
<td>Q1</td>
</tr>
<tr>
<td>FDA Filing</td>
<td>Potential Approval Oct 26th</td>
<td>Launch ²</td>
</tr>
</tbody>
</table>

1: FDA decision expected at so-called PDUFA date set to October 26th, 2023
2: Expected through partner Catalyst
3: Staggered launch with first country Germany
4: Launch review by NICE (National Institute for Health and Care Excellence) evaluation
Vamorolone in Becker muscular dystrophy
Becker muscular dystrophy (BMD) disease profile and corticosteroid use

<table>
<thead>
<tr>
<th>Genetics</th>
<th>Cause</th>
<th>Patients</th>
<th>Symptoms</th>
<th>Medical need</th>
</tr>
</thead>
<tbody>
<tr>
<td>X-linked recessive form of muscular dystrophy typically diagnosed between age 5 and 15</td>
<td>Partial loss of function of dystrophin with a broad clinical variability</td>
<td>Higher life expectancy and lower prevalence than DMD (approx. 1/3)</td>
<td>Progressing muscle weakness and degeneration with later and slower onset compared to DMD</td>
<td>No approved treatment and under-represented development efforts</td>
</tr>
</tbody>
</table>

**CORTICOSTEROIDS IN BMD**
Steroid use is lower compared to DMD due to perceived less favorable benefit-risk ratio for current steroids.¹
Vamorolone addresses safety concerns and may qualify for a chronic treatment in BMD

**Evidence for corticosteroid use in BMD**
- Efficacy from limited patient case studies
- Data from *in vivo* models of inflammation

![Pie charts showing current use of corticosteroids in BMD and DMD](chart.png)

1 Cowen et al., 2019, BMC Neurology
Vamorolone holds promise in BMD based on data generated for DMD

Vamorolone potential benefits in BMD\(^1,2\)

1. Anti-inflammatory agent with reduced side effects via dissociative character of vamorolone
2. Cardiac benefit via mineralocorticoid antagonism
3. Potential to increase dystrophin levels via suppression of dystrophin-targeted microRNAs

CURRENT CLINICAL DEVELOPMENT IN BMD (all three drugs are developed both in BMD and DMD)\(^3\)

- **Phase 2 completed**: Givinostat (Italfarmaco), 12-month treatment in 51 patients
- **Phase 2 recruiting**: EDG-5506 (Edgewise), 12-month treatment in 54 patients
- **Phase 2 recruiting**: Vamorolone (ReveraGen/Santhera), 24-week treatment in 39 patients
- **Natural history study ongoing**: (Edgewise), 24-month observational study in 150 patients

---

1: Heier et al. Life Science Alliance Feb 2019, 2 (1);2: Fiorillo et al., 2018, PhysiolGenomics; 3 Clinicaltrials.gov RCT: Randomized controlled trial; PI: Principle investigator
Lonodelestat in cystic fibrosis and potentially other inflammatory pulmonary disorders
Cystic fibrosis is a rare genetic lung disorder with unmet medical need

<table>
<thead>
<tr>
<th>Genetics</th>
<th>Cause</th>
<th>Patients</th>
<th>Symptoms</th>
<th>Medical need</th>
</tr>
</thead>
<tbody>
<tr>
<td>Autosomal recessive disorder diagnosed at young age</td>
<td>Mutations in the CF transmembrane conductance regulator (CFTR) gene</td>
<td>More than 80,000 patients in US and Europe combined</td>
<td>Persistent lung infections, chronic inflammation and loss of respiratory function</td>
<td>No approved treatment specifically addressing inflammation in CF</td>
</tr>
</tbody>
</table>

Need to break vicious cycle of airway obstruction, respiratory failure and resulting chronic inflammation\textsuperscript{1,2}
Lonodelestat targets elastase, a protease responsible for lung damage

Pathological levels of neutrophil elastase (NE) during inflammation destroy lung tissue over time\(^1\)

Lonodelestat is a highly potent, reversible and selective NE inhibitor

- Effective in pico-molar range (Ki 0.05nM) inhibiting free and membrane bound NE
- Demonstrated efficacy in various in vivo models for lung diseases (inhaled/intranasal)

Administration via inhalation using Pari eFlow®

- CE marked medical device since 2005, widely used in chronic indications, also in CF
- High prolonged exposure in lung but desired low systemic exposure after inhalation (1000:1)

\(^1\) Polverino et al. CHEST 2017; 152(2):249-262;
Successful Phase 1 program paves way for further clinical development

Key achievements in CF development program
- Safe dose regimen identified
- Effect on inflammatory biomarker established
- High local targeting through inhalation demonstrated

Opportunities beyond CF
- Excessive neutrophil activity in range of pulmonary diseases provides rationale for pipeline expansion
- Identified opportunities in both acute and chronic indications
- Program is Phase 2 ready in CF and ARDS, but currently paused

Next steps in CF
- Preparation of Phase 2a program in patients currently non-eligible for CFTR modulator therapy with a dose of 2 x 40 mg daily

Opportunities beyond CF
- Acute lung injury / ARDS
- Pulmonary arterial hypertension
- Primary ciliary dyskinesia
- Non-cystic fibrosis bronchiectasis
- Alpha-1 antitrypsin deficiency
- Chronic obstructive pulmonary disease
- Pulmonary fibrosis following cancer therapy
- ...and other disorders associated with excessive elastase activity

ARDS: Acute respiratory distress syndrome; CFTR: Cystic Fibrosis Transmembrane Conductance Regulator
Financial overview & upcoming milestones

<table>
<thead>
<tr>
<th>Key figures* – year ending Dec 31, 2022</th>
<th>CHF million</th>
</tr>
</thead>
<tbody>
<tr>
<td>• Net (loss) for the period</td>
<td>(71.1)</td>
</tr>
<tr>
<td>• Cash (used) in operations</td>
<td>(29.8)</td>
</tr>
<tr>
<td>• Cash &amp; cash equivalents</td>
<td>1.3</td>
</tr>
<tr>
<td>• Debt outstanding (maturity 2024)</td>
<td>(43.2)</td>
</tr>
<tr>
<td>• Shareholders' equity</td>
<td>(43.7)</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Cash runway</th>
</tr>
</thead>
<tbody>
<tr>
<td>• On closing of North America License into Q1-2025</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Capital structure – June 19, 2023</th>
</tr>
</thead>
<tbody>
<tr>
<td>Listed SIX (SANN)</td>
</tr>
<tr>
<td>• Basic shares outstanding(see note) 104,808,418</td>
</tr>
<tr>
<td>• Market capitalization           CHF 91.8 million</td>
</tr>
<tr>
<td>• Major shareholders Catalyst 11.3% ; Idorsia 10.4%</td>
</tr>
<tr>
<td>• Research coverage H.C. Wainwright, ValuationLAB</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Upcoming milestones – vamorolone</th>
</tr>
</thead>
<tbody>
<tr>
<td>Q3-2023 Early access program France / UK</td>
</tr>
<tr>
<td>Q4-2023 • US FDA decision (PDUFA date Oct 26)</td>
</tr>
<tr>
<td>• EU EMA &amp; UK approval decision</td>
</tr>
<tr>
<td>• UK approval decision</td>
</tr>
<tr>
<td>Q1-2024 • US commercial launch by Catalyst</td>
</tr>
<tr>
<td>• EU, UK commercial launch by Santhera</td>
</tr>
</tbody>
</table>

*All amounts audited Consolidated IFRS
Note on shares outstanding = Excludes treasury and includes 14,146,882 to be issued to Catalyst on closing of license
Santhera Pharmaceuticals

Developing medicines to meet the needs of patients living with rare diseases

June 2023