



# Santhera Pharmaceuticals Investor Presentation

May 2025

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# Santhera Pharmaceuticals

A fully integrated  
commercial stage  
biopharmaceutical  
company

## SIX Swiss Exchange listed company (SANN)

- Global headquarters near Basel (Switzerland)
- About 110 employees; will remain <150 employees

## AGAMREE® is a differentiated product in Duchenne muscular dystrophy (DMD)

- A unique dissociative corticosteroid which maintains powerful anti-inflammatory properties of traditional steroids but with an improved safety profile

## Global rollout underway – positive market reception

- Approvals by five authorities (U.S., EU, UK, CN, HK)
- Own commercialization of AGAMREE in Western European countries, with first launches in 2024 in Germany and Austria
- Launched in the U.S. by partner Catalyst

## Financing in place for next steps

- New financing in August 2024 with gross funding up to CHF 69 Mio
- Cash runway to cash-flow break-even in mid 2026
- Cash at the 31 December 2024 of CHF 40.9 Mio

# DMD is lifelong neuromuscular disorder characterized by progressive loss of muscle strength and function

1. **No cure** and high medical need
2. **Onset at age 3-5 years** and life expectancy in the late 20s to mid-30s
3. **Progressive muscle weakness** needing chronic treatment
4. **Loss of ambulation** in early teenage years followed by respiratory failure and cardiac complications



# Current therapies with intrinsic limitations: too late - too little - too soon

## Today's standard of care:

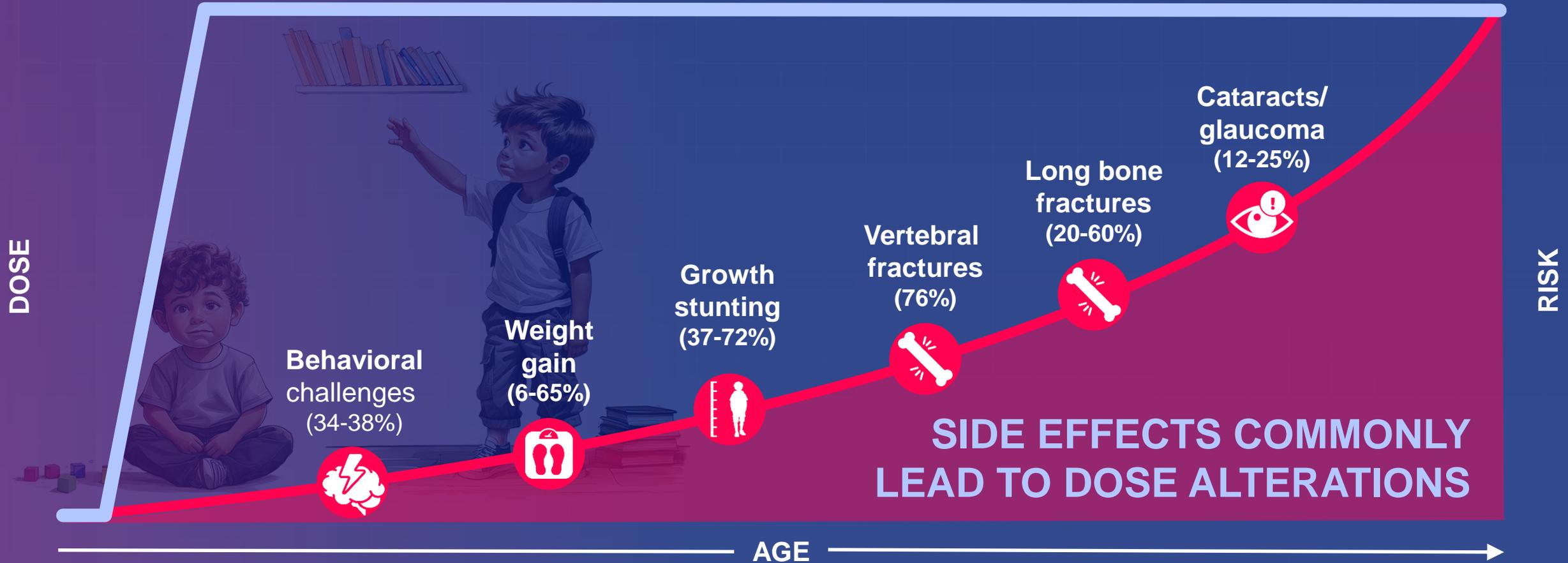
- Corticosteroids are the standard of care in combination with other treatments
- Corticosteroids can delay disease progression by 2-3 years
- Corticosteroids have limitations due to serious side-effects

## Today's problem:

- Start too late
- Dose too little
- Stop too soon



# Managing side effects and improving tolerability remain key challenges with traditional corticosteroids



1. Cowen L, et al. BMC Neurol. 2019;19:84; 2. Wong B, et al. J Pediatr. 2017;182:296–303; 3. Bello L, et al. Neurology. 2015;85:1048–55; 4. Guglieri M, et al. JAMA. 2022;327(15):1456–68; 5. Weber DR, et al. Pediatr. 2018;142(Suppl 2):S43–52; 6. Zhang T, Kong X. Exp Ther Med. 2021;21(5):447; 7. Osorio AN, et al. Neurología. 2019;34(7):469–81. 8. Rice ML, et al. J AAPOS. 2018;22:192–6; 2. Angelini C. Muscle Nerve. 2007;36:424–35. 9. Ward LM, et al. Pediatrics. 2018;142:S34–42; 10. Ward LM. Front Endocrinol (Lausanne). 2020;11:576.

# AGAMREE® (vamorolone)

## A better foundational therapy

### AGAMREE addresses limitations of standard corticosteroid therapy

- Retained anti-inflammatory action and efficacy
- Reduction of steroid-associated side effects related to:
  - growth
  - bone health
  - behavior
- May have additional benefits – Heart health

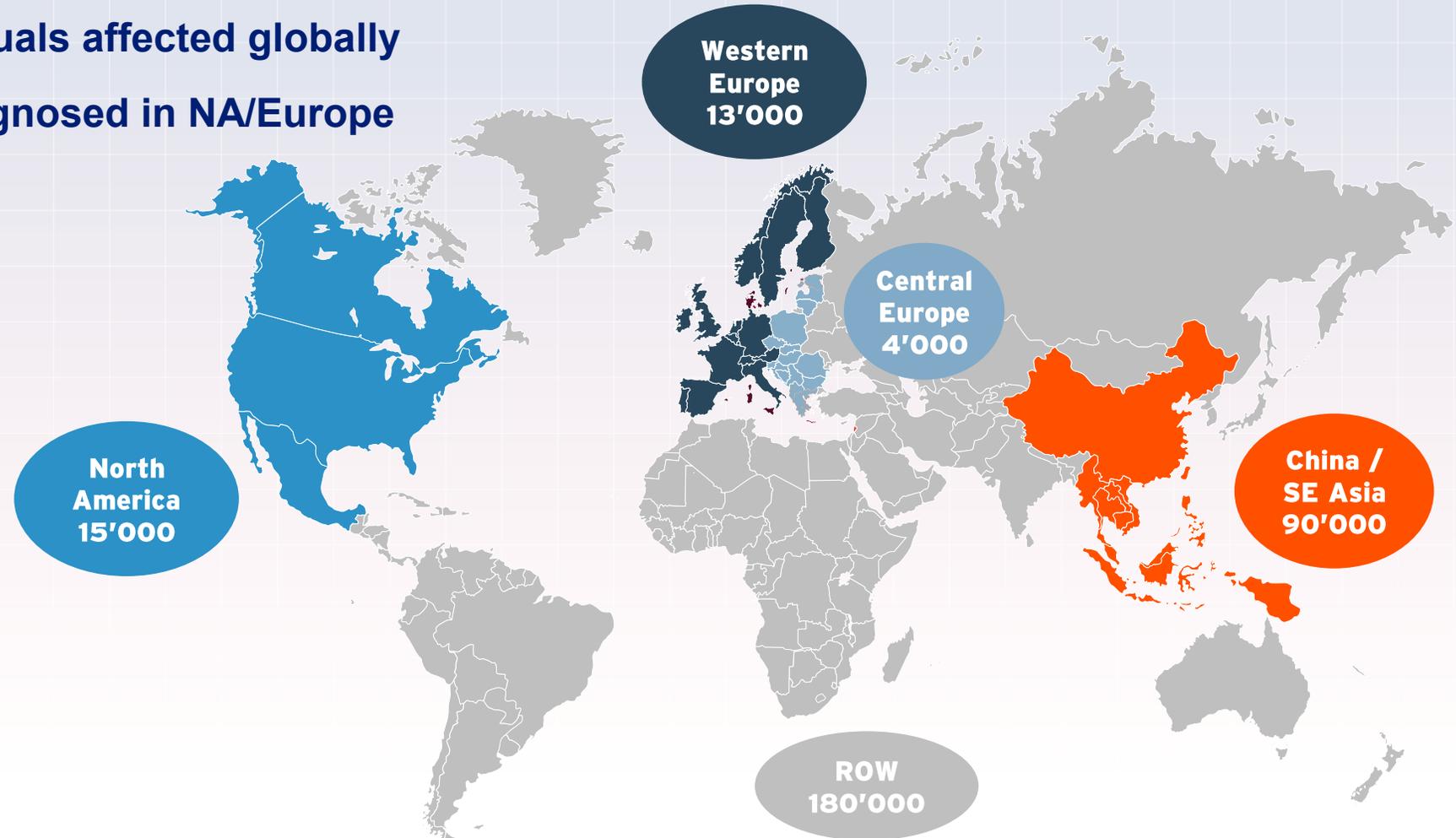
### AGAMREE allows patients to stay:

- On time
- On dose
- On treatment

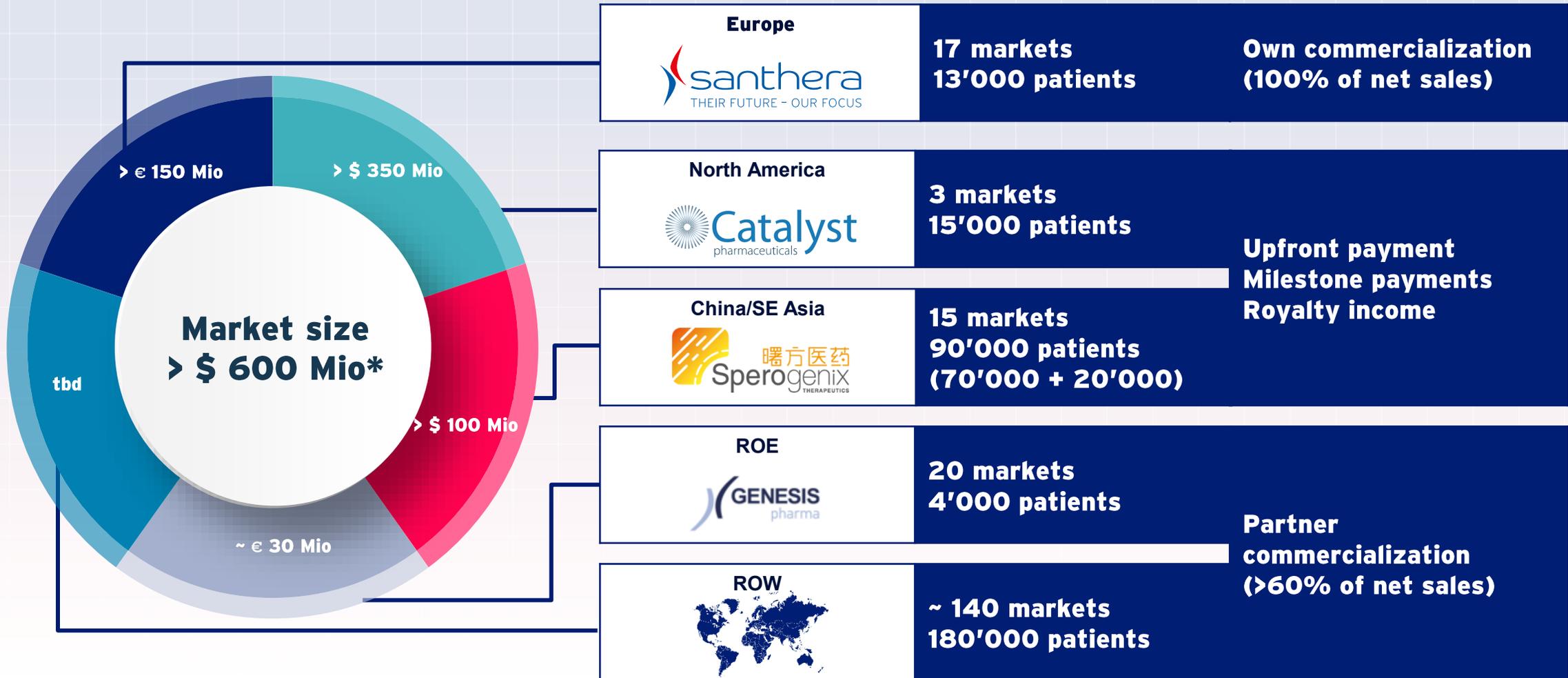


# DMD is one of the largest rare disease markets with a clearly defined patient group

- Around 300'000 individuals affected globally
- 90% of patients are diagnosed in NA/Europe
- 50-75% of patients on steroid treatment
- Patients are treated in specialized centers
- HCPs familiar with steroid usage



# Global DMD market opportunity with substantial runway for growth



\* Santhera estimates

# Operational highlights - Year ending 31 Dec 2024 (including post period)

1

## Successful launch progress in own markets:

- In Germany and Austria almost 30% of patients on steroid treatment switched to AGAMREE within the first 12 months

2

## Other EU direct markets progressing well:

- UK NICE positive recommendation, Dec 2024
- UK full launch, Q2 2025
- Further launches expected through 2025 and H1 2026

3

## Catalyst successfully launched in the US (Mar)

- Reported 2024 sales of USD 46 Mio, surpassing guidance
- 2025 revenue guidance: USD 100 - 110 Mio, would trigger a further milestone payment to Santhera

4

## Sperogenix partnership in China:

- Early access program commenced during 2024, approval Dec 2024
- Commercial rollout expected mid 2025 on a non-reimbursed basis

5

## Rollout in other territories:

- Additional distribution agreements signed to expand into non-direct European markets, Israel and Qatar
- Named patient supply agreement signed with Clinigen covering territories with no commercially available supply

6

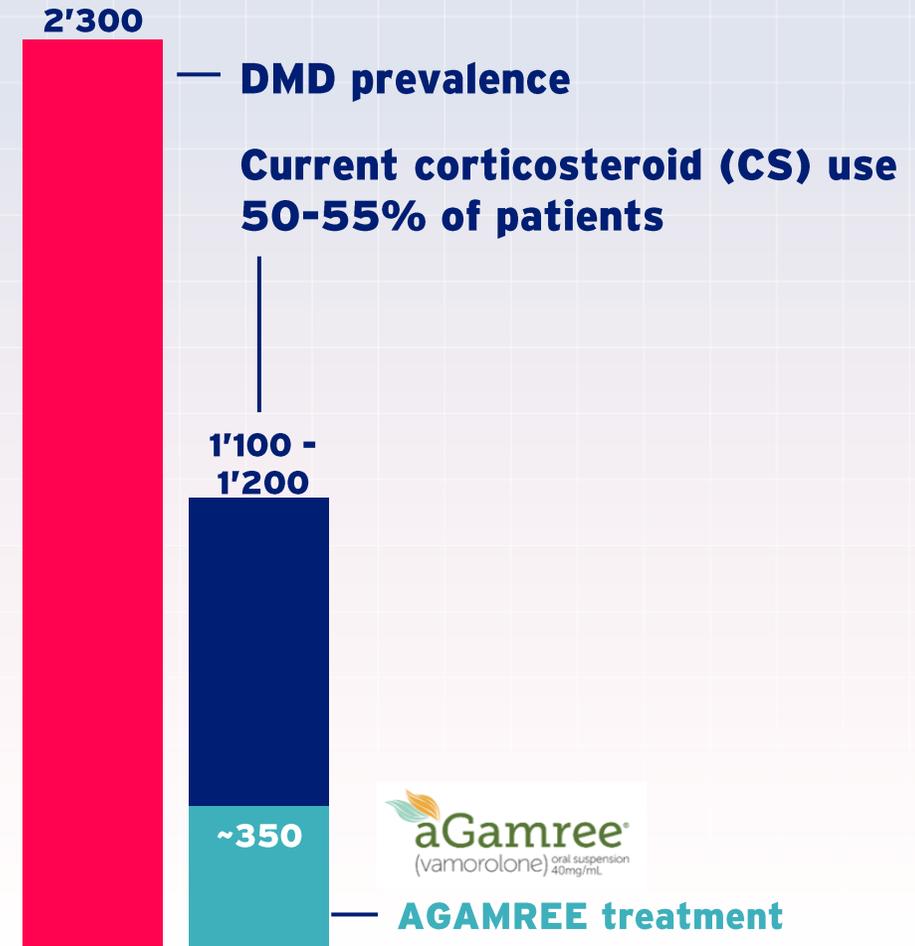
## Manufacturing expansion:

- Santhera and Catalyst validating second manufacturers
- To increase capacity, streamline supply chain efficiencies, and reduce manufacturing costs

# Rapid adoption of AGAMREE<sup>®</sup> by patients and payers in Germany & Austria

## Successful 1<sup>st</sup> year of AGAMREE launch

- **30% share of steroid treated patients with AGAMREE** in Germany and Austria after one year
  - newly diagnosed aged 4-5
  - switchers aged 6-12
- **No** clinical trial sites/experience prior to launch
- Federal **price** in Germany **EUR 3'612.50** (per 100ml bottle) as per German formula
- Germany reference market for several other countries

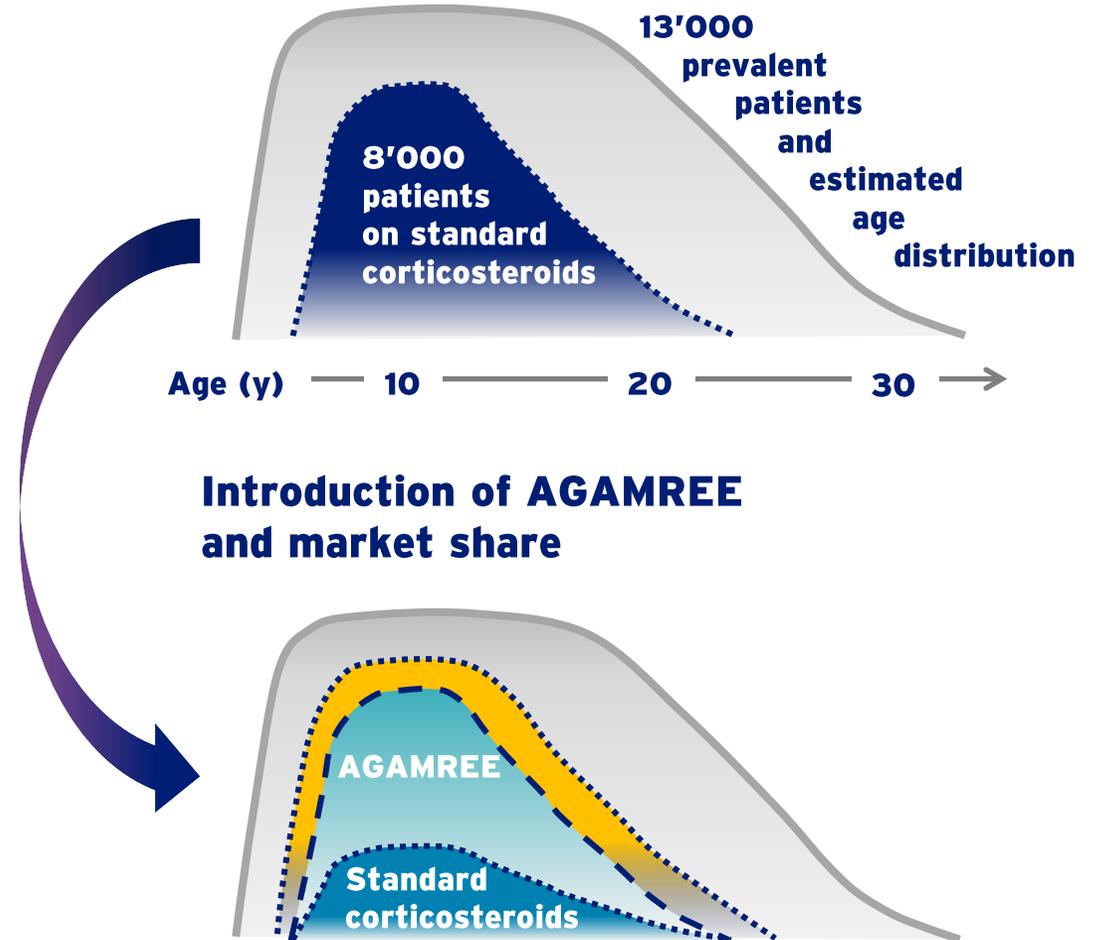
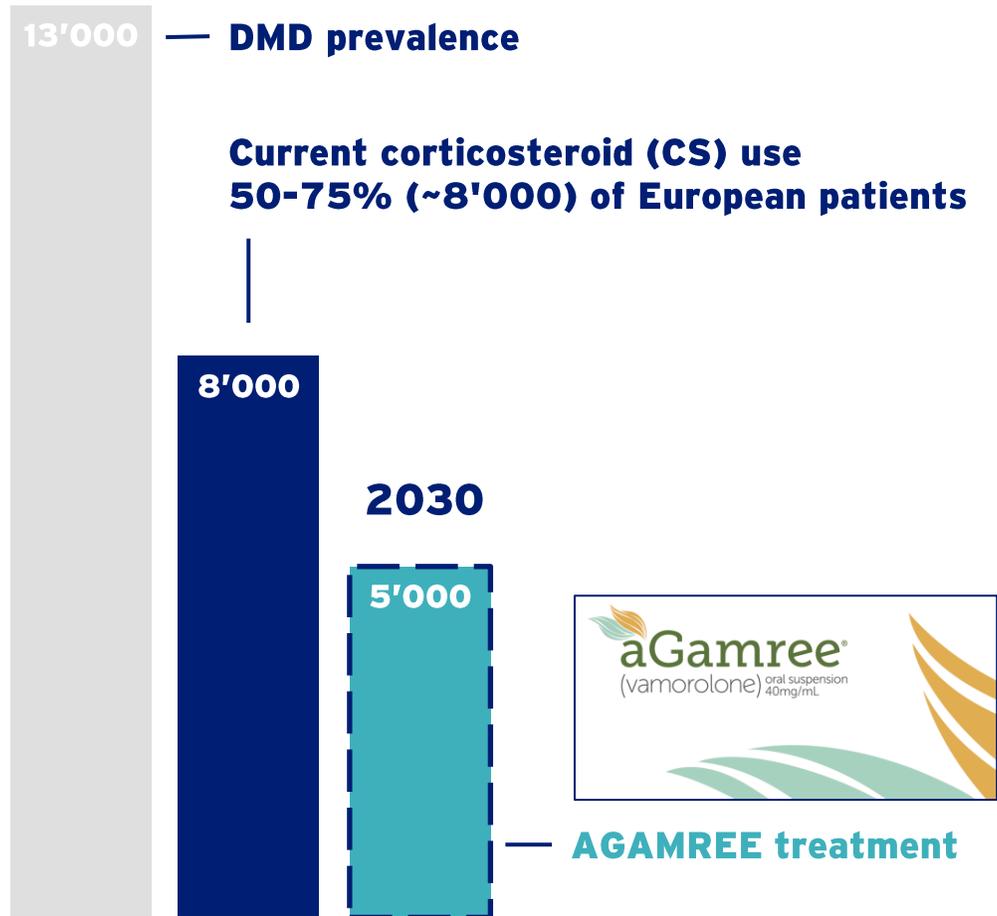


# Key European launches progressing as planned

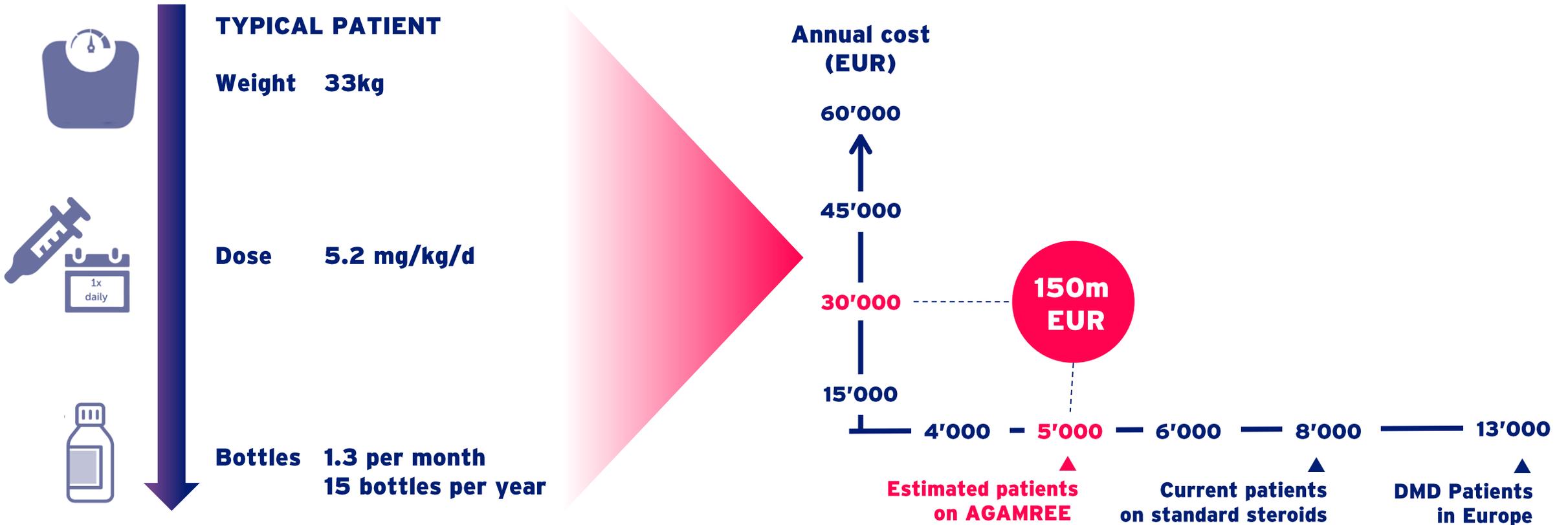
			2024				2025				2026	
		Status	Q1	Q2	Q3	Q4	Q1	Q2	Q3	Q4	H1	H2
PHASE 1	Germany / Austria	Launched	Launch	Pricing negotiations			✓					
	UK	Launched	Pricing negotiations			✓		Launch				
PHASE 2	Spain	Submitted		NPP*	Pricing negotiations							
	Italy	Submission Q1 2025				NPP*	Pricing negotiations					
	Nordic	In preparation					Pricing negotiations					
	Benelux	In preparation		NPP*			Pricing negotiations					
PHASE 3	France	Submitted	Pricing negotiations							TBD		
	Switzerland	Reg. submitted				Regulatory submission and pricing & reimbursement						
	Other Europe	Ongoing	Launch preparations									

\*Named Patient Program

# Targeting 5'000 patients on AGAMREE® in Santhera European territory by 2030



# Expected peak sales of EUR >150 million in Santhera territory in 2030



Assumption based on patients treated, average weight, dose and price per bottle

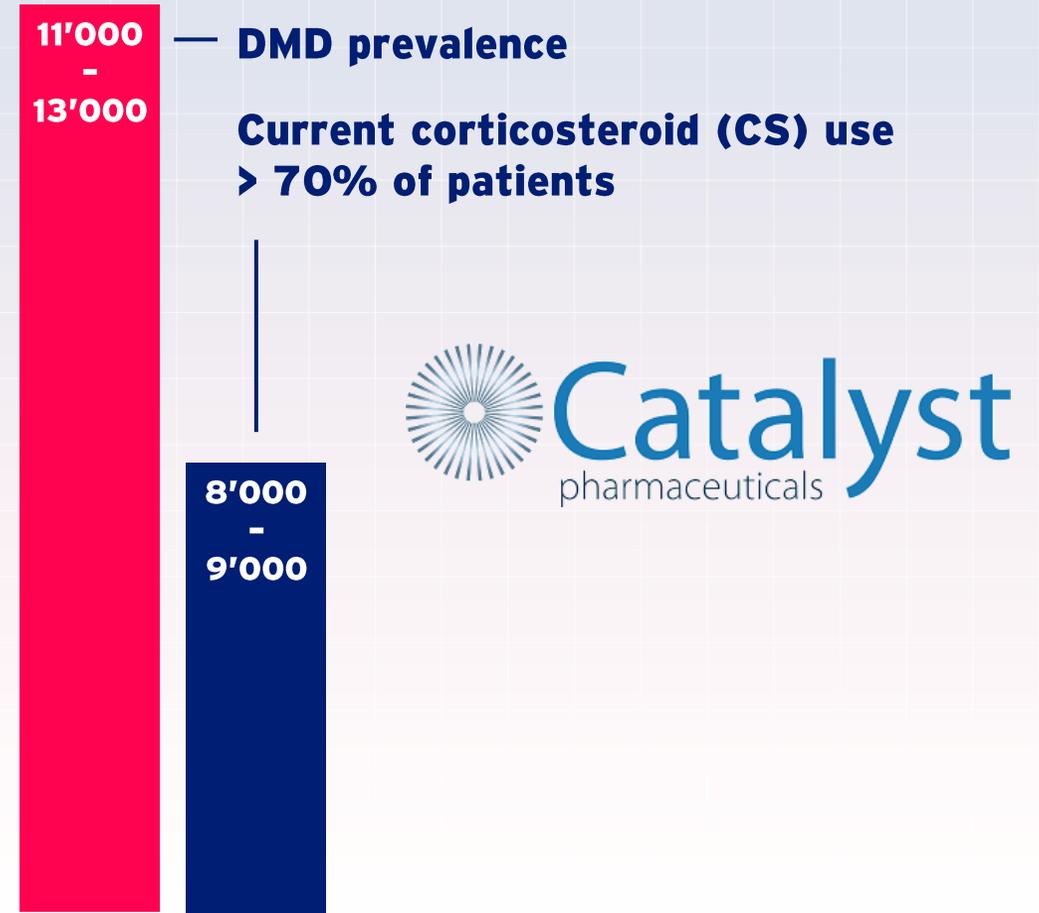
# Successful US launch in March 2024 beating initial calendar year guidance

## Rapid uptake in 3 quarters of AGAMREE® launch (2024) year

- Net sales of **USD 46 Mio** outperform initial guidance of **USD 25-30 Mio**

## 2025 Guidance:

**Net sales of USD 100-110 Mio**



# China / SE Asia progressing as planned



## NMPA approval received for AGAMREE® in China in December 2024 - earlier than expected

- AGAMREE incorporated into both the Priority Review Program and the Breakthrough Therapy Program
- Hong Kong approval followed 9 days later in the same month

## Soft Launch planned for H2/2025 in China

➤ 100 patients on AGAMREE as part of Hainan EAP program launched in June 2024.



# We are expanding Manufacturing capacity

## Additional manufacturing sites in development:

- **Q1 2026:** First supply ready from second CMO
- **Ensures supply** for geographical expansion
- **Provides redundancy** and security of supply
- **Streamlines** supply chain & reduces lead time
- **Reduces** manufacturing cost and working capital

Catalyst Pharmaceuticals evaluates second manufacturer in US

Sperogenix Therapeutics plans for local manufacturing until 2029 (latest)



# Financial Highlights - Year Ending 31 Dec 2024

1

**Total Revenues: CHF 39.1 Mio** (2023: CHF 103.4 Mio)  
Driven by revenue from strong product sales in Germany and Austria, offset out by significant licensing milestones recognised in 2023 from out-licensing activities.

2

**Product sales: CHF 14.8 Mio** (2023: CHF 0.8 Mio)  
Driven by the successful launch of AGAMREE in Germany and Austria.

3

**Royalties & milestones: CHF 19.3 Mio** (2023: CHF 99.9 Mio)  
2023 revenues were bolstered by out-licensing milestones received from Catalyst Pharmaceuticals in the U.S. and Sperogenix in China.

4

**Revenue from supply of product and services to partners: CHF 5.0 Mio** (2023: CHF 2.7 Mio)  
Product sold to licensing partners increased as US launch commenced.

5

**Operating expenses: CHF 57 Mio** (2023: CHF 32 Mio)  
2023 was positively impacted by net gain of CHF 17.0 Mio on sale of the idebenone business. Excluding this gain, 2024 operating expenses were 15% higher YoY.

6

**Operating loss: CHF 33.2 Mio** (2023: CHF 68.8 Mio profit)  
2023 was positively impacted by significant out-licensing milestones from Catalyst and Sperogenix as well as the net gain on the sale of the idebenone business.

7

**Financing: CHF 69 Mio secured**  
In August, the Company secured up to CHF 69 Mio in royalty and debt financing through a new term loan (CHF 35 Mio) and royalty monetization agreement (USD 30 Mio).

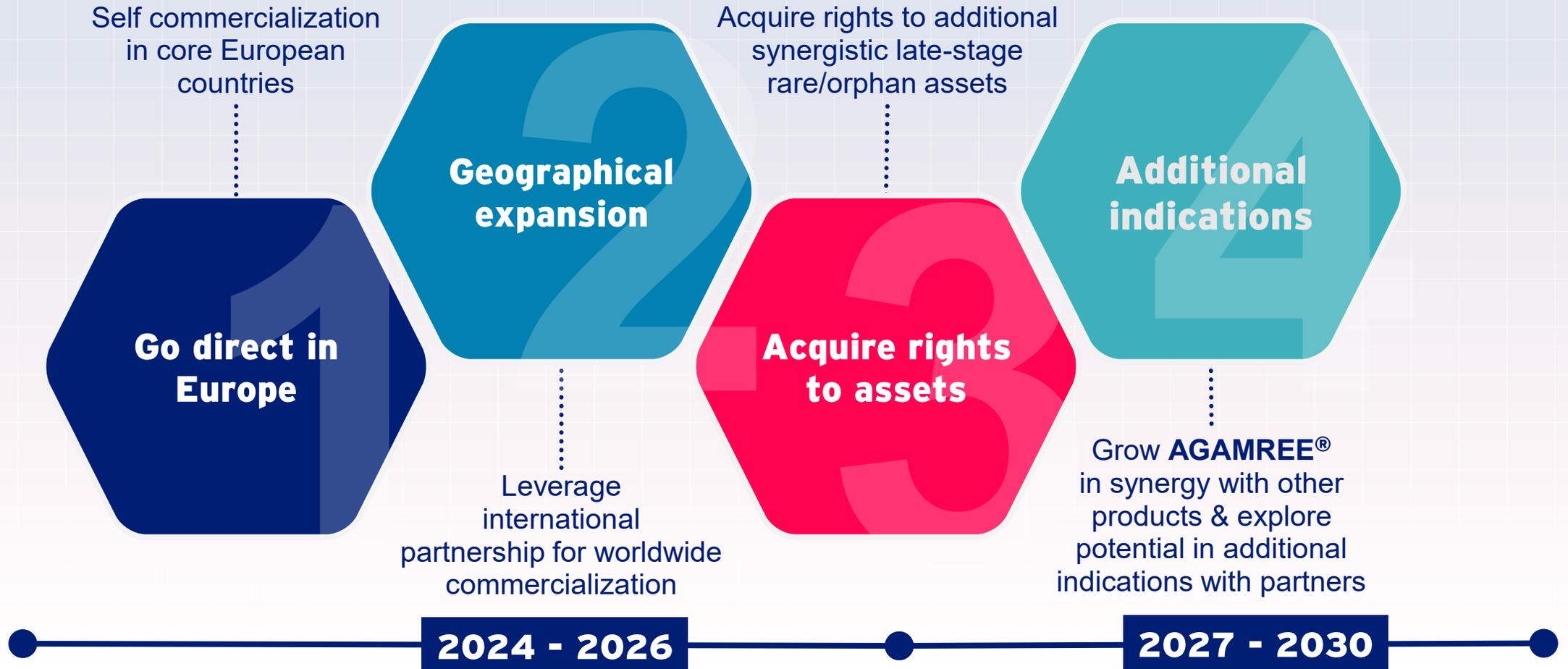
8

**Cash and cash equivalents: CHF 40.9 Mio** (2023: CHF 30.4 Mio).  
Cash runway extended to mid-2026 at which point the Company expects to be cash break-even.

# Financial guidance

- **2025 revenue guidance:** CHF 65-70 Mio
- **2028 revenue outlook:** EUR 150 Mio – this covers direct and partnered markets, as well as royalty income from North America and China, excludes potential milestones payments received from partners
- **2030 revenue outlook (direct markets):** Expect > EUR 150 Mio of sales in own direct markets (excludes distributor and licensed market revenues/royalties)
- **Operating expenses (SG&A and R&D) 2025 and going forward on constant portfolio basis:** CHF 50-55 Mio – this excludes non-cash share compensation

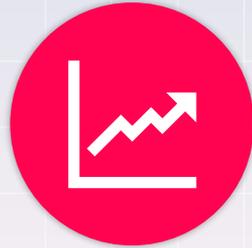
# Clear strategy with four pillars of revenue generation



# We have everything in place to successfully serve the DMD market



**A differentiated product with worldwide rights**



**A clear growth strategy**



**A strong & growing partner network**



**A nimble organization with expertise**



**Funded to projected cash-breakeven**

**Thank you**

**For your time**

**APPENDIX**

# Further geographic expansion targeted

**Santhera is actively pursuing further international partnerships with focus on:**

**Opportunity for additional mid-to long-term revenue and profitability with limited investment**

## **Step 1:**

- Turkey
- GCC
- Brazil/LatAm

## **Step 2:**

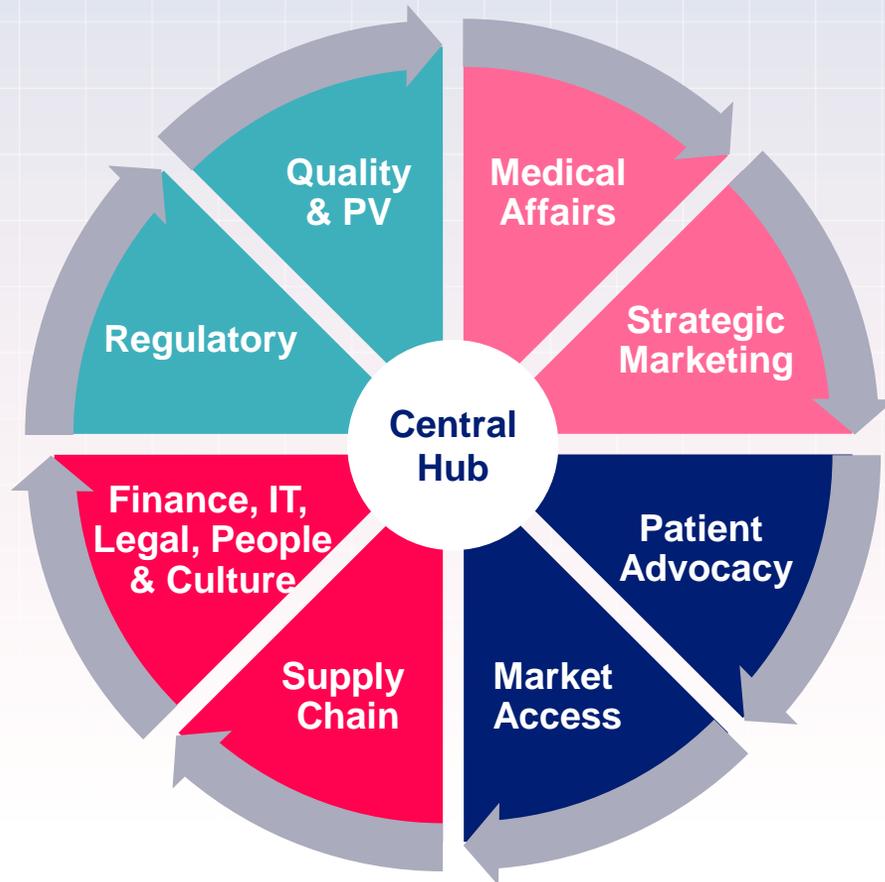
- S. Korea
- Australia/NZ
- India
- Japan
- Russia tbd

**We are prioritizing countries based on market opportunity.**



# Nimble commercial set-up supports markets

Headquarters functions support own country teams, licensing and commercialization partners



## License Partners

- Catalyst (North America)
- Sperogenix (Greater China/SEA)

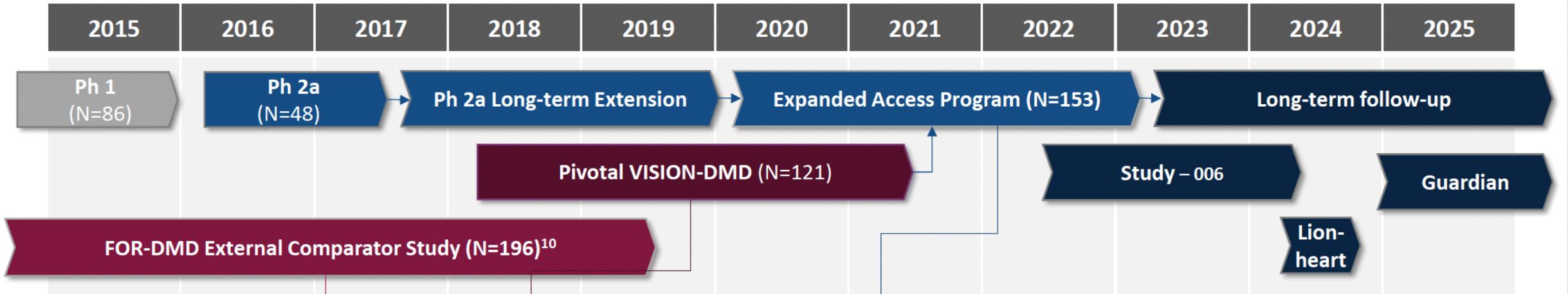
## Santhera

- Germany, Austria, Switzerland
- United Kingdom, Ireland
- France
- Italy
- Spain, Portugal
- Benelux
- Nordics

## Commercial Partners

- Genesis (20 European countries)
- Megapharm (IL), ASTE (Qatar), Clinigen (International/Named Patient)

# Our data builds on over 200 patient-years exposure from more than 200 boys treated for up to 7 years



## External comparison

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

## Pivotal study

establishes efficacy vs placebo, comparable to prednisone (at week 24), maintenance of effect (at week 48) and safety differentiation in patients 4 to <7 years of age

## 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

### Lionheart:

mechanistic study of mineralocorticoid receptor antagonism in human

### Guardian Study:

open-label long-term extension with yearly readout and end in 2028

# Financing: August 2024 Gross funds c. CHF 69 Mio



## Term Loan: (Highbridge Capital Management)

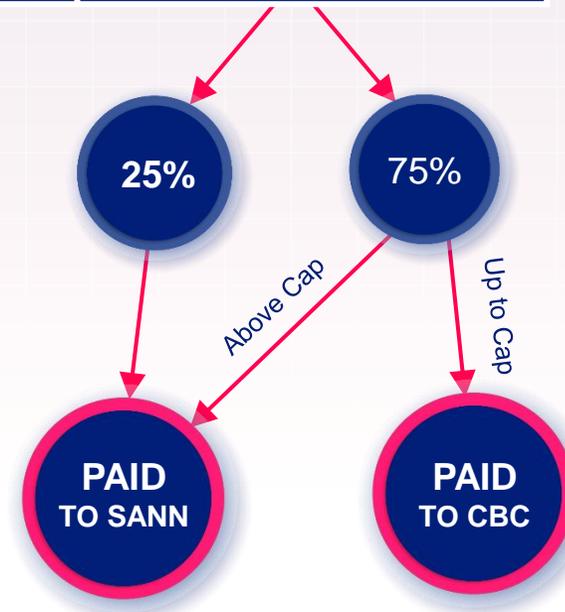
- Received CHF 35 Mio from a 4-year term loan: Interest rate of 3-month SARON Plus 9.75% 1st two years interest only, remainder 15% pa amortization, bullet repayment Aug 28
- Existing CHF 7 Mio private convertible bond with a strike of CHF 10 extended by 12 mo. to Aug 25
- 237k new warrants issued with a strike price of CHF 11.1

## Royalty Monetization Agreement: (R-Bridge - Affiliate of CBC Group)

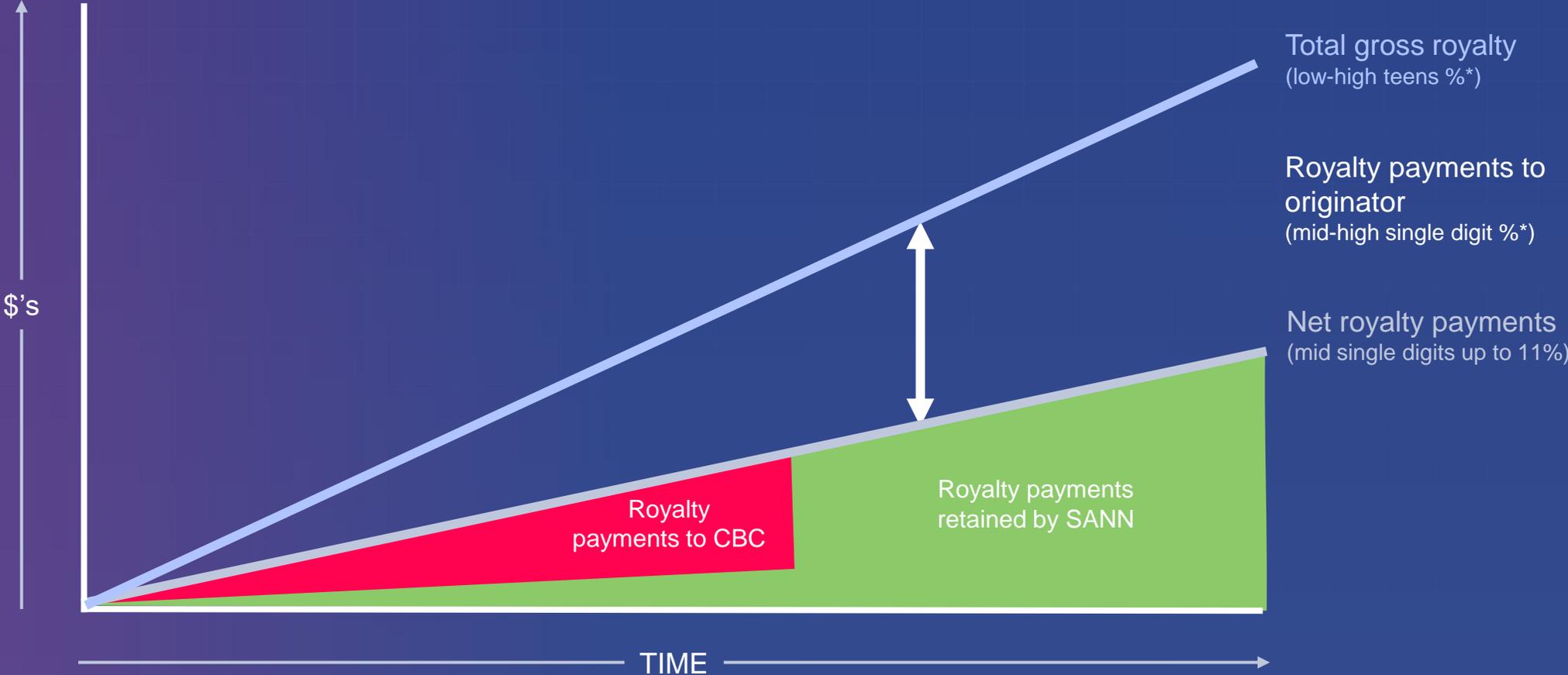
- Received \$30 Mio through a royalty monetization agreement, plus potentially will receive up to a further \$8 Mio based on China sales related milestones
- Royalty agreement is partial and capped: Relates to 75% of future net royalty income streams from agreements with Catalyst (US) & Sperogenix (China)
- Once cap (based on the \$30 Mio) is met, all royalty payments revert to Santhera
- Santhera retains certain rights to buy back the royalty income stream
- Milestones received from either Catalyst and Sperogenix are excluded from the CBC agreement and continue to be fully received by Santhera

# Summary revenue/royalty stream

	Direct Markets (Western Europe)	Distributors (E Europe & other)	Licensed Catalyst / Sperogenix	
Revenues Booked to SANN	100%	>60%	Low-high teens % gross royalty*	Booked Revenue Guidance 2028: EUR 150 Mio
Royalty payment to originator (in COGS line)	(less mid-high single digit % royalty*)	(less mid-high single digit % royalty*)	(less mid-high single digit % royalty*)	
	<b>Net direct revenues</b>	<b>Net distributor revenues</b>	<b>Net Royalties</b>	
	Booked Revenue Guidance 2030 > EUR 150 Mio			



# Summary royalty stream



\* For sales <\$500 Mio, for sales >\$500 Mio additional royalties are booked but also paid away to originator

# Share Capital and Major Shareholders

## Share Capital - as of 1<sup>st</sup> March 2025

	Number '000	Comments
Listed shares outstanding	13,514	
Less Treasury	(642)	
<b>Basic shares outstanding</b>	<b>12,872</b>	
<b>Dilution</b>		
Convertible bonds	697	CHF 6,971k Maturing Aug 2025 at a strike of CHF 10
Warrants	237	237k at strike CHF 11.10 458k at strike CHF 20.00 – not included
Employee Schemes	576	Vested
<b>Total dilution</b>	<b>1,510</b>	
<b>Diluted shares outstanding</b>	<b>14,382</b>	

## Major shareholders > 5%

- Catalyst Pharmaceuticals: 11.0 %
- Idorsia Pharmaceuticals: 7.3 %

# Executive Management Team



**Dario Eklund**  
Chief Executive Officer



**Catherine Isted**  
Chief Financial Officer



**Dr. Oliver P.  
Kronenberg**  
Chief Legal Officer and  
Secretary to the Board



**Dr. Shabir Hasham**  
Chief Medical Officer



**Marc Schrader**  
Chief Technology Officer



**Dr. Geert-Jan van  
Daal**  
Chief Commercial Officer