Santhera Pharmaceuticals

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

Corporate Presentation

March 2025

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.

Santhera Pharmaceuticals Corporate Snapshot

SIX Swiss Exchange listed company (SANN)

Global headquarters near Basel (Switzerland) with internationally experienced leadership team

Own commercialization of lead asset in Western European countries

Strong rare disease development capabilities

AGAMREE® (vamorolone) in Duchenne muscular dystrophy

Differentiated safety profile addresses needs across broad DMD patient segments

Potential as alternative to corticosteroids in range of other therapeutic indications

Approvals by five authorities (US, EU, UK, CN, HK)

FDA (10/2023), EMA/EC (12/2023), MHRA (01/2024) and NMPA/NPC in China/Hong Kong (12/2024)

First launches in 2024 in Germany and Austria by Santhera and in U.S. by partner Catalyst

Positive market reception: patients on Agamree in Germany/Austria reach 30% of corticosteroid patents in the first year

Finance

New financing in August 2024

Cash runway to cash-flow break-even including commercial EU infrastructure & launch

Major shareholders: Catalyst, Idorsia and Highbridge Capital

santhera
THEIR FUTURE – OUR FOCUS

Value driver in DMD with broad potential

AGAMREE® (vamorolone) foundational therapy in DMD

- U.S. FDA full approval on October 26, 2023; US launch on March 13, 2024
- EC full approval on December 18, 2023; German launch on Jan 15, 2024
- MHRA full approval on Jan 2024 and by NMPA/NPC in China/Hong Kong in Dec 2024
- Potential as alternative to corticosteroids in broad range of therapeutic areas
- Own commercialization in selected Central & Western EU countries
- Peak potential > EUR 150 million in DMD (Santhera estimate for own markets)
- Commercialization by partner Genesis in remaining EU and Eastern European countries and Megapharm in Israel
- Additional global expansion to be achieve through further partnering/distribution agreements

Worldwide rights for all indications
(Licensing partners in North America and China/ SE Asia)

aGamree® (vamorolone) oral suspension 40mg/mL

santhera
THEIR FUTURE - OUR FOCUS

Lead asset AGAMREE® in DMD approved by FDA, EMA, MHRA and NMPA

Molecule	Study / Indication	Proof of Concept	Pivotal	Filing	Market	Phase 4	Remarks
Vamorolone • dissociative steroid • oral suspension	DMD development VISION-DMD	Approved in US, EU, UK and CN/HK					North America & China/ South-East Asia partnerships with Catalyst and Sperogenix, respectively
	DMD long-term extension GUARDIAN	Ongoing					Establish long-term benefit in DMD for patients on drug for 6+ years
	Mechanistic study LIONHEART	Completed					Established mineralocorticoid receptor antagonism in human
Life cycle management	Becker muscular dystrophy	Q4 / 2025					Trial under FDA grant to partner ReveraGen
	Steroid alternative in rare pediatric indications	H1 / 2026					Plans to be disclosed

santhera
THEIR FUTURE – OUR FOCUS

DMD offers attractive opportunity in well-defined orphan disease market

The DMD indication with few current treatment options is a fast-growing multi-billion market

- Approx. 30,000 – 35,000 patients in U.S. 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

Focused expert centers treating patients in EU and U.S.

DMD	Centers	HCPs
U.S.	~90	~450
EU4+UK	~180	~750

Current and new therapies likely to be used in combination with corticosteroids

- Exon skippers and read through therapies serve niche segments based on genetic mutation[1]
- Gene therapies deliver micro-dystrophin partially restoring function with re-dosing challenges[1]

1: Approved in US only

santhera THEIR FUTURE - OUR FOCUS

AGAMREE® can fill the need for a better foundational therapy in DMD

Corticosteroids delay disease progression by 2-3 years, but associated toxicities limit their use

ESTABLISHED 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 SOON

VAMOROLONE OFFERING

Subtle difference in steroid chemical structure leads to dissociative properties.

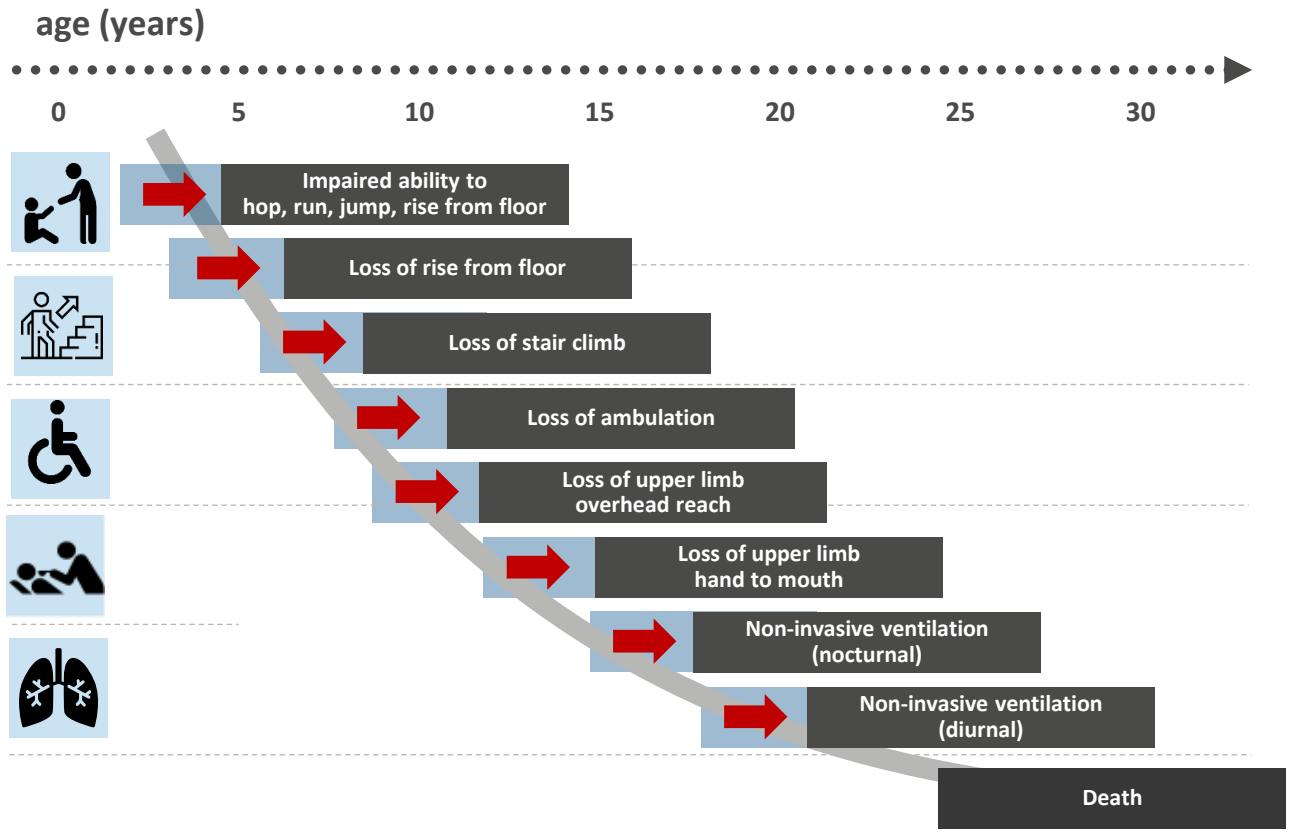
Maintained anti-inflammatory efficacy with improved safety profile has been established.

NEW DISSOCIATIVE STEROID

santhera
THEIR FUTURE - OUR FOCUS

Corticosteroids delay disease progression in DMD by 2 – 3 years[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[1-4]
- Early initiation of corticosteroids preserves muscle function and strength, delaying time to loss of functional milestones by 2 – 3 years[4,6]
- Steroid treatment associated with a reduction in all-cause mortality, new onset and progressive cardiomyopathy[5]

1. Birnkrant et al. (2018) Lancet Neurology, 1474; 2. Cowen et al. BMC; Neurology (2019) 19:84; 3. Asher et al. (2020) Exp. Opin. Bio. Therapy, 20:3, 263; 4. McDonald CM et al., Lancet 2018, 3391 (10119):451-461; 5. Schram et al; PCL Cochrane Database of Systematic Reviews (2013), 61(9);948-54; 6. Matthews et al Cochrane Database of Systematic Reviews (2016)

Corticosteroid treatment is associated with well-defined toxicities

…up to 65% of DMD patients discontinue treatment early due to adverse events[1-3]

37%  **Growth failure/delay[2,3]**

37%  **Behavioral changes[3]**

30%  **Bone changes or fractures[2,3]**

55%  **Cushingoid appearance[3]**

65%  **Weight gain[3]**

31%  **Excessive hair growth[2,3]**

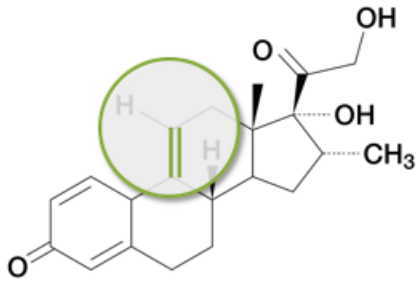
19%  **Cataracts[2]**

1. Cowen L, et al. BMC Neurol. 2019;19:84; 2. Wong B, et al. Treatment Pediatr. 2017;182:296–303;
3. Bello L, et al. Neurology. 2015;85:1048–1055;

santhera
THEIR FUTURE - OUR FOCUS

AGAMREE® (vamorolone) dissociative properties

Subtle but impactful difference in chemical structure separates vamorolone from classical steroids[1-5]



Signature double bond impacts receptor binding and alters enzyme and membrane interactions

Like corticosteroids, efficacy maintained by potent anti-inflammatory action

- Retained inhibition of NF-κB pro-inflammatory transcription factor

Unlike corticosteroids, potential for reduction of steroid-associated side effects

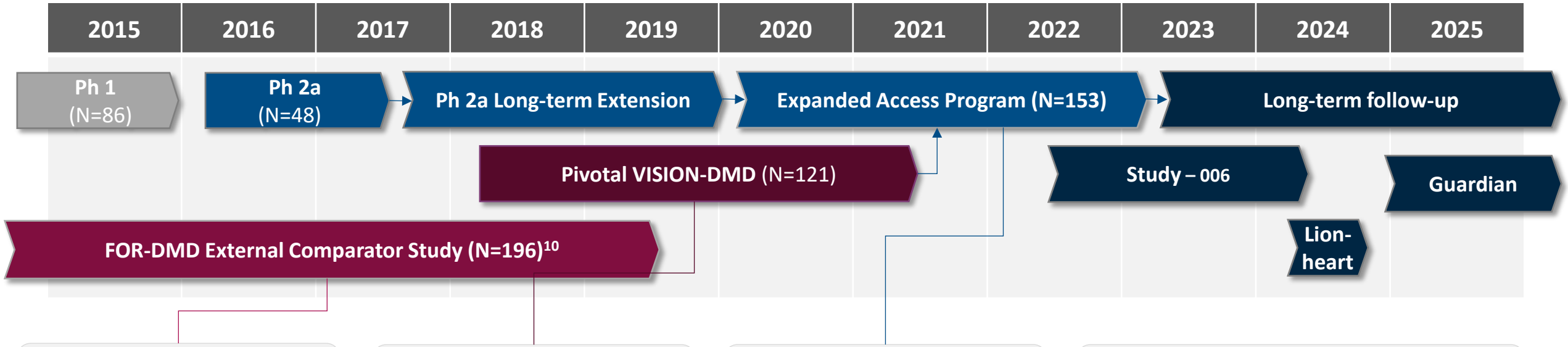
- Less activation of genes related to side effects
- Not a substrate of hydroxysteroid dehydrogenase
- Potent mineralocorticoid antagonist (eplerenone-like)
- Membrane stabilizer

NF-κB=nuclear factor kappa B., 1. Smith et al. PLOS Medicine. (2020); 2. Dang et al. MDA Abstr. #47 (2021) 3. Guglieri M Poster EP 524 WMS 2021, 4. Heier CR, et al. EMBO Mol Med. 2013;5:1569-1585, 5. Liu X, Proc Natl Acad Sci U S A. 2020 Sep 29;117(39)

santhera
THEIR FUTURE - OUR FOCUS

Comprehensive AGAMREE® (vamorolone) development [2-9]

200 patient-years exposure in 160 DMD boys treated with vamorolone for up to 7 years[1]



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

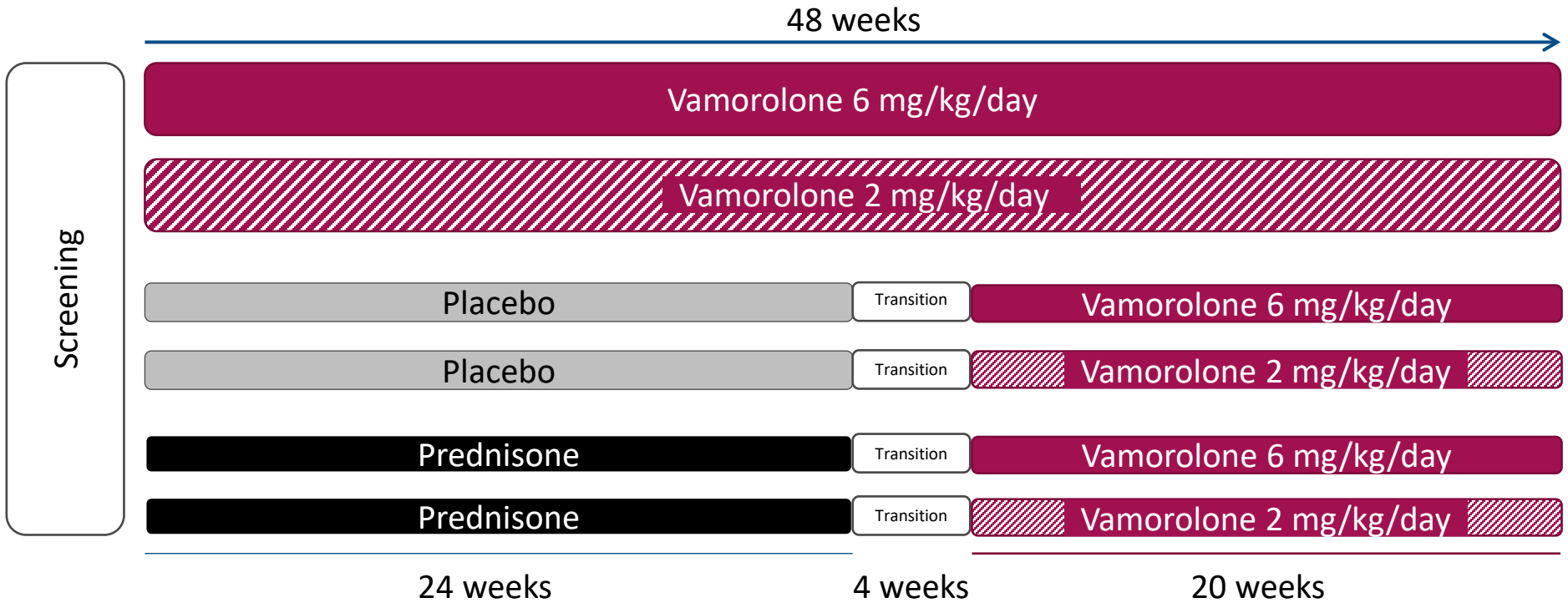
Study-006:
supportive data in patients 2-18 years of age
Lionheart:
mechanistic study of mineralocorticoid receptor antagonism in human
Guardian Study:
open-label long-term extension with yearly readout and end in 2028

1. Data on File VAM-2021-001, 2. Hoffman et al. Steroids (2018); 3. Conklin et al. Ph. Res. (2018); 4. Hoffman et al. Neurology. (2019); 5. Smith et al. PLOS Med. (2020); 6. Mah et al, JAMA Open Network 2022; 7. Mavroudis et al. J. Clin. Ph. (2019); 8. Li et al. J. Clin Ph. (2020); 9. Liu et al. PNAS (2020), 10. Guglieri et al JAMA 2020; * Santhera Data on File; MRA: Mineralocorticoid receptor antagonism

santhera
THEIR FUTURE - OUR FOCUS

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

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)

santhera THEIR FUTURE - OUR FOCUS

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

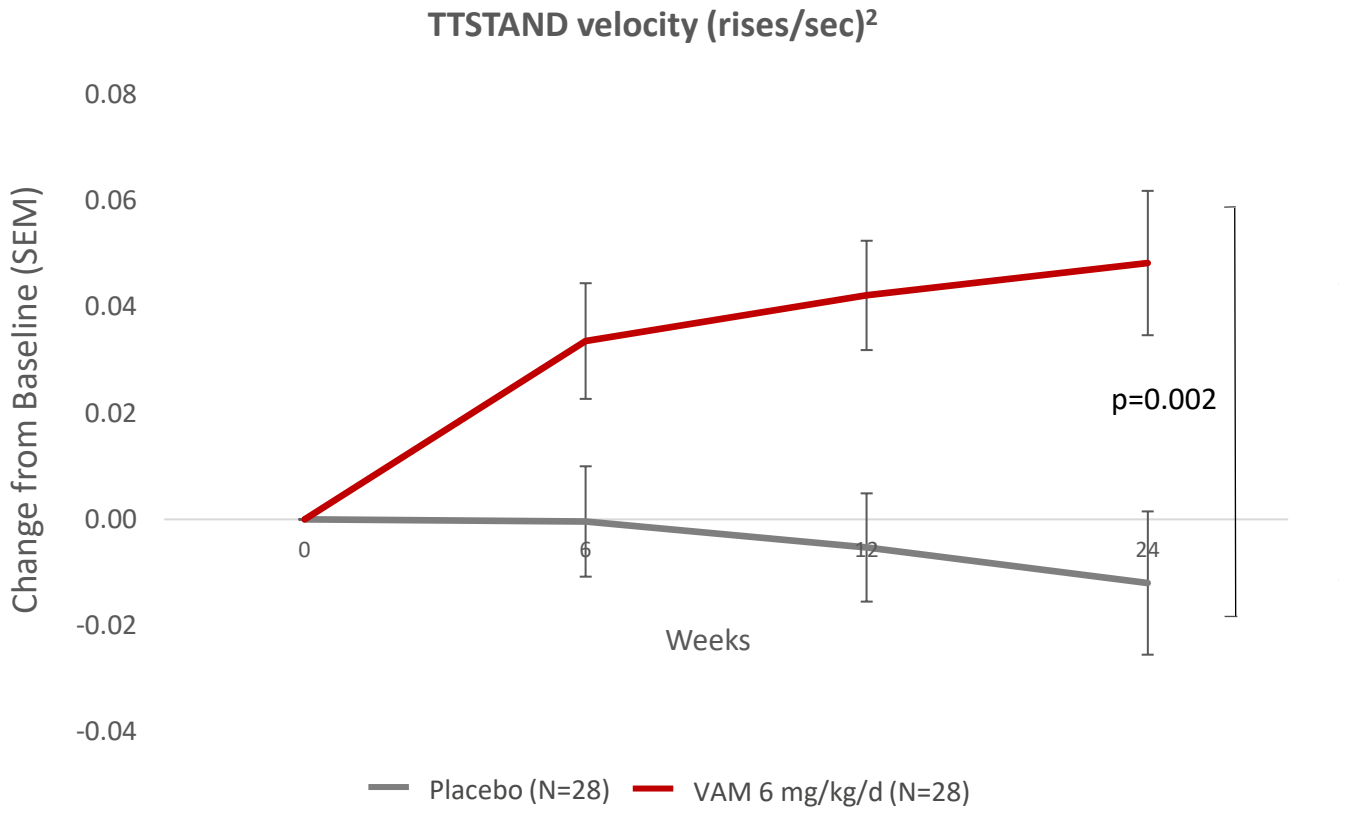
Rank	Endpoint	Comparison vs placebo	Difference	MCID	P-value
Primary	**TTSTAND velocity**	**vam 6mg/kg**	**0.06 rises/s**	**>0.023 rises/s[1]**	**0.002**
Pre-Specified, Hierarchical Secondary	TTSTAND velocity	vam 2mg/kg	0.04 rises/s	>0.023 rises/s[1]	0.017
	6MWT	vam 6mg/kg	42 m	>26-32 m [2,3]	0.003
	6MWT	vam 2mg/kg	37 m	>26-32 m [2,3]	0.009
	TTRW velocity	vam 6mg/kg	0.24 m/s	>0.2 [1,2] m/s	0.002
	TTRW velocity	vam 2mg/kg	0.13 m/s	>0.2[1,2] m/s	0.103
Exploratory	TTCLIMB velocity	vam 6mg/kg	0.07 task/s		<0.001
	TTCLIMB velocity	vam 2mg/kg	0.06 task/s		0.006
	NSAA	vam 6mg/kg	3.4 points	>2-3 points [4,5]	<0.001
	NSAA	vam 2mg/kg	3.2 points	>2-3 points [4,5]	<0.001

1. Guglieri JAMA 2020; 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). mITT-1; MMRM estimates of changes from baseline to week 24, all doses daily.1. Duong et al J Neuromuscul Dis. 2021; 8(6):939-48; 2. McDonald et al, Muscle Nerve. 2013; 48(3):357-68; Henricson et al 2013; 4. Wong et al Neuromuscular Disorders. 2019; 29:S106.; 5. Haberkamp et al Neuromuscul Disord. 2019; 29(7):514-6; MCID: Minimum clinical important difference

santhera
THEIR FUTURE - OUR FOCUS

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]



23% improvement in time to rise after 6 months of treatment with VAM 6mg/kg/d[3]

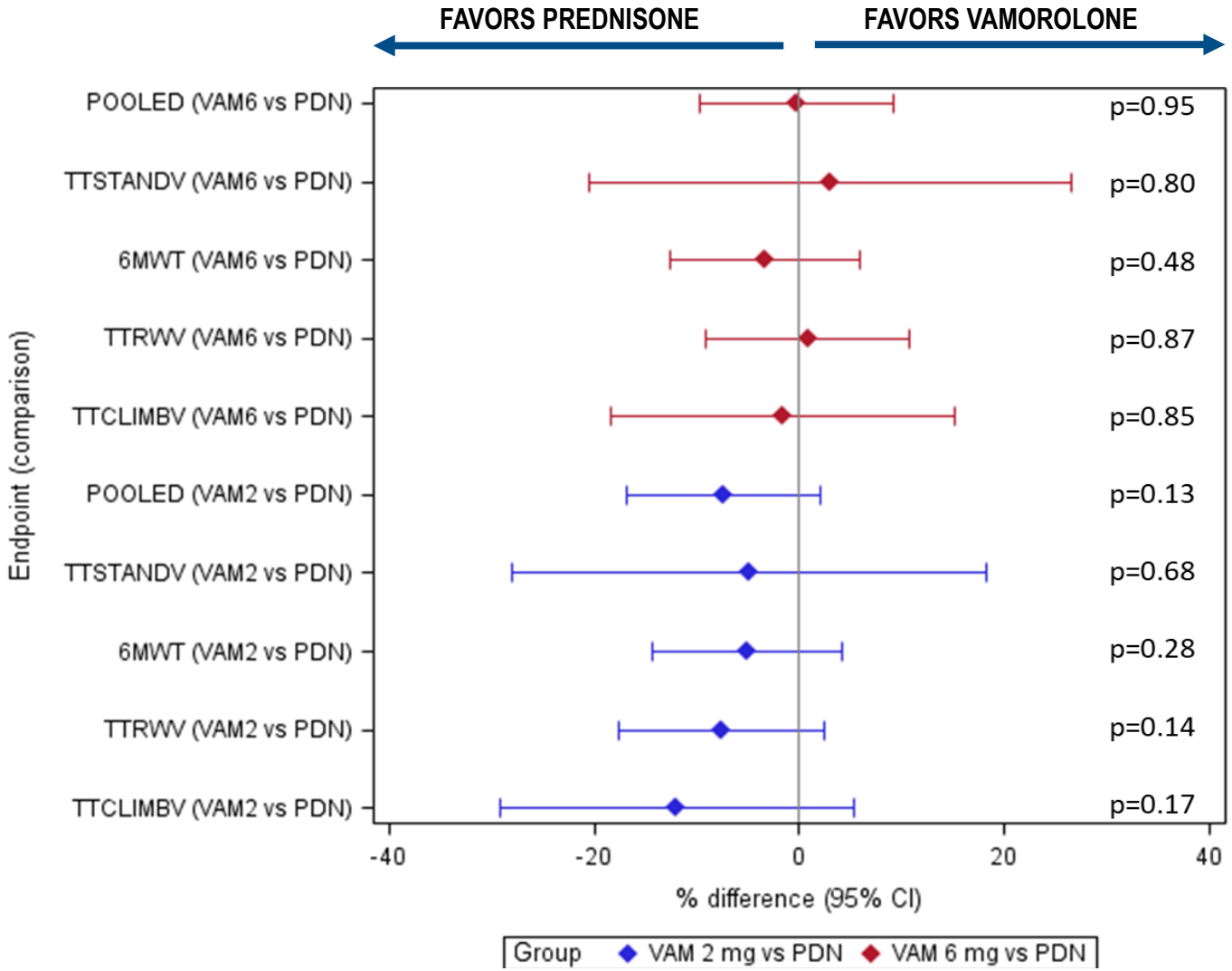
Rise time (sec) [2]	BL	w 24	% Change
VAM 6 mg/kg/d	6.0	4.6	- 23%
Placebo	5.4	5.5	+ 2%

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

santhera
THEIR FUTURE - OUR FOCUS

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)

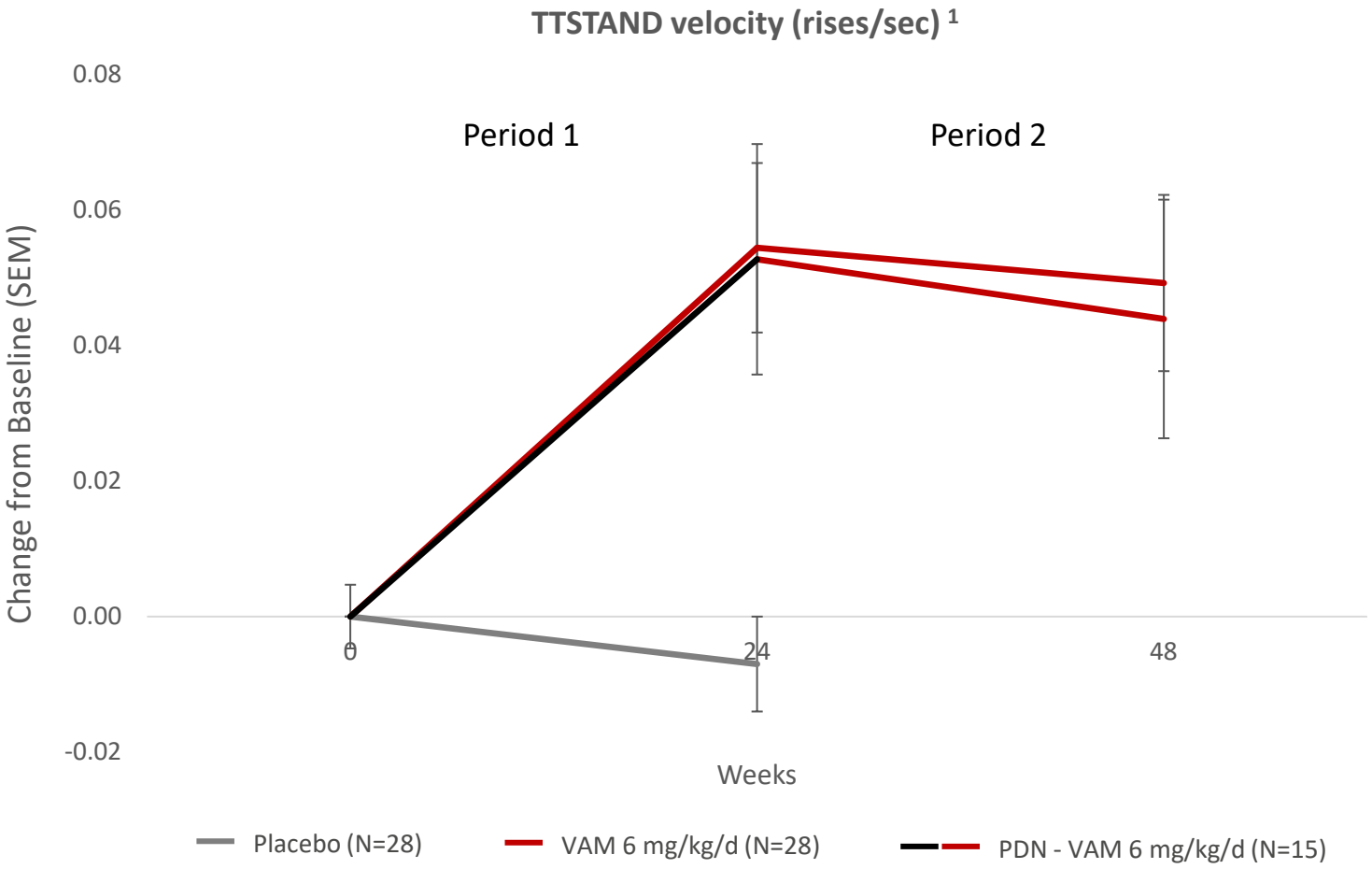


PDN: Prednisone 0.75 mg/kg/d; VAM: Vamorolone at 2 and 6 mg/kg/d; Time to Stand (TTSTAND), 6 Minute Walk Test (6MWT), Time to Run/Walk 10m (TTRW), Time to Climb 4 Stairs (TTCLIMB), North Star Ambulatory Assessment (NSAA).
Data on file (adapted from Poster 524 presented at WMS 2021), mITT-1

santhera
THEIR FUTURE - OUR FOCUS

No loss of efficacy when switching from prednisone to vamorolone

Durable treatment effect maintained over 48 weeks with vamorolone 6 mg/kg/d[1]



- 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[2]

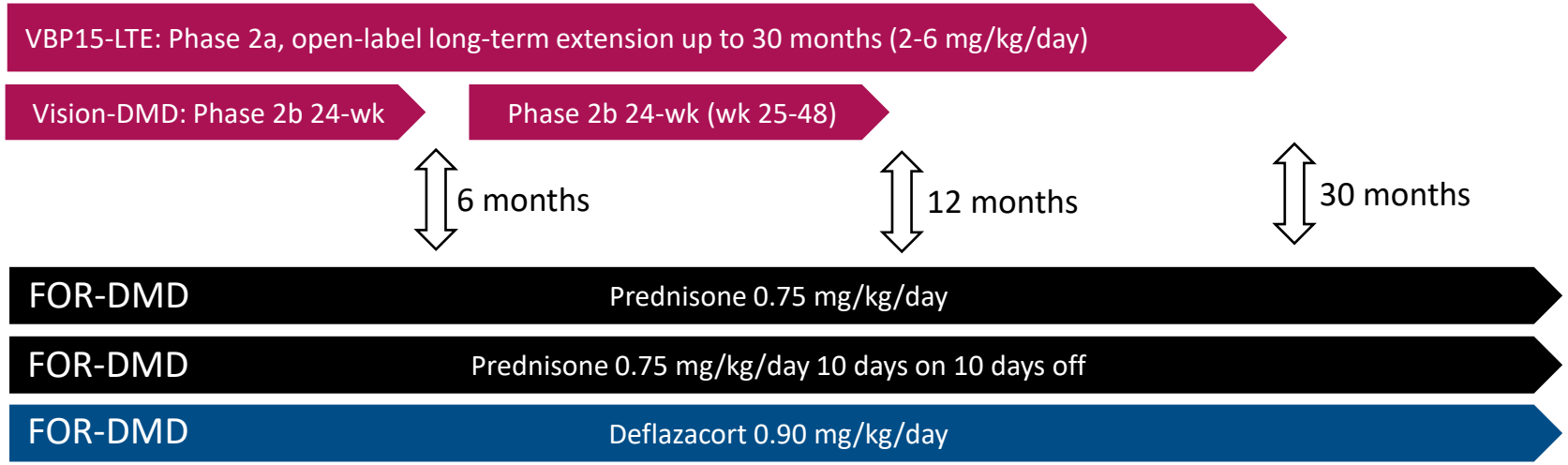
1. Data on File VAM-2021-002, mITT-2: modified intention to treat population from period 1 and 2, MMRM estimates of changes from baseline. PDN: prednisone 0.75mg/kg/day, PDN-VAM: prednisone 0.75 mg/kg/d in Period 1 transitioned to vamorolone 6mg/kg/d in Period 2 group after a 4-week tapering period;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

The FOR-DMD study provides external comparator data[1]

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

DMD boys 4- <7
Steroid-naive
N=121 (pivotal Phase 2b, 48-wks)
N=46 (LTE, 30-months)

DMD boys 4-8
Steroid-naive
N=196 , 3-5 year follow-up



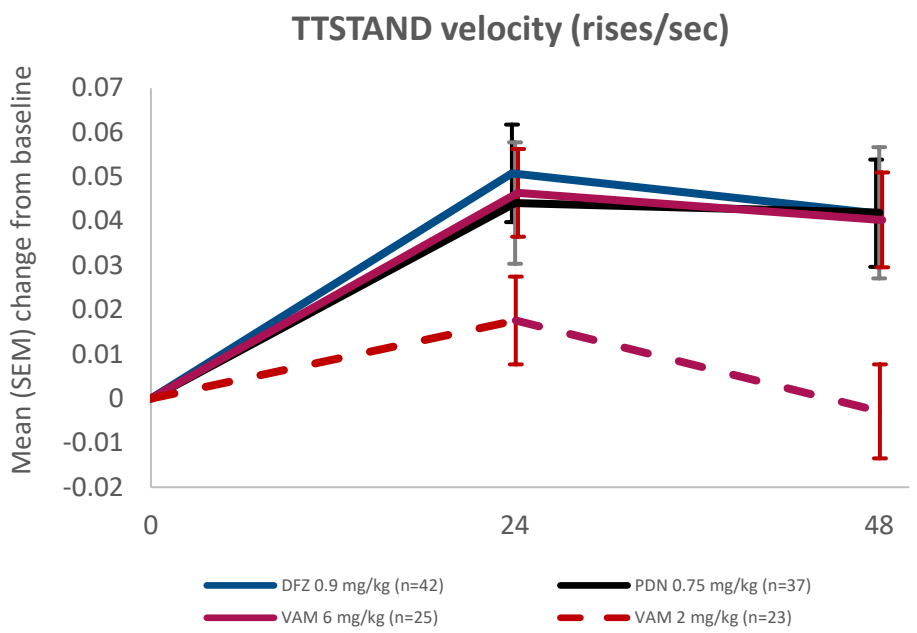
Time point	Efficacy		Safety	
	Comparison	Method	Comparison	Method
24 weeks / 6 months	PDN (VISION-DMD) vs PDN (FOR-DMD)	Propensity score matching[2]	PDN (VISION-DMD) vs PDN (FOR-DMD)	Inclusion criteria matching[3]
48 weeks / 12 months	VAM vs PDN vs DFZ	Propensity score matching[2]	VAM vs PDN vs DFZ	Inclusion criteria matching[3]
2.5 years[4]	Not applicable	Not applicable	VAM vs PDN vs DFZ	Inclusion criteria matching[3]

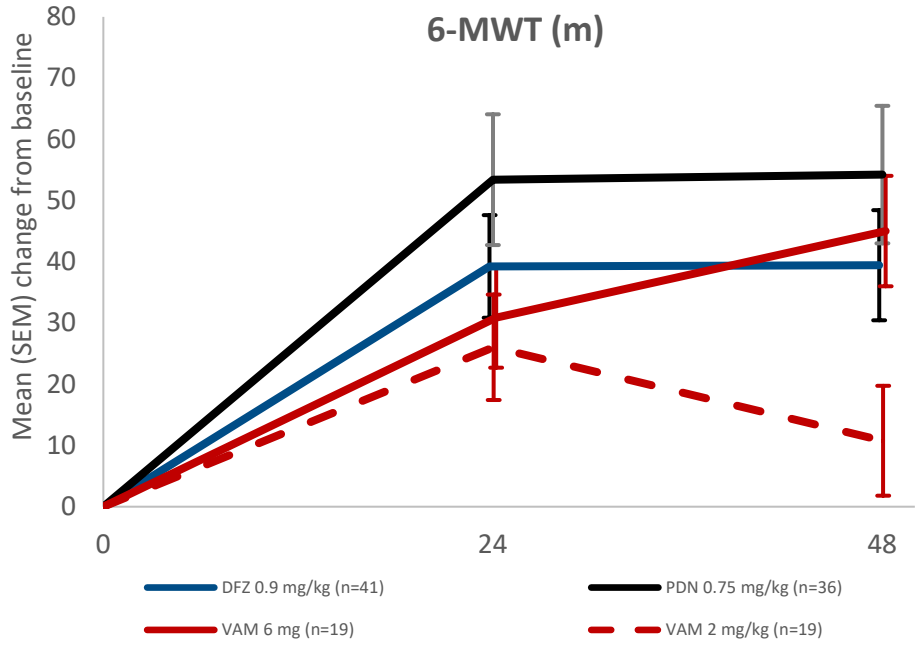
1. Guglieri et al JAMA 2022 doi:10.1001/jama.2022.4315, 2. Pre-defined propensity scores calculated based on baseline age, TTSTAND, NSAA score, height and weight; analysis weighted by the propensity scores.Patients meeting the common inclusion criteria of all studies are included 3. For safety endpoints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

santhera
THEIR FUTURE - OUR FOCUS

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

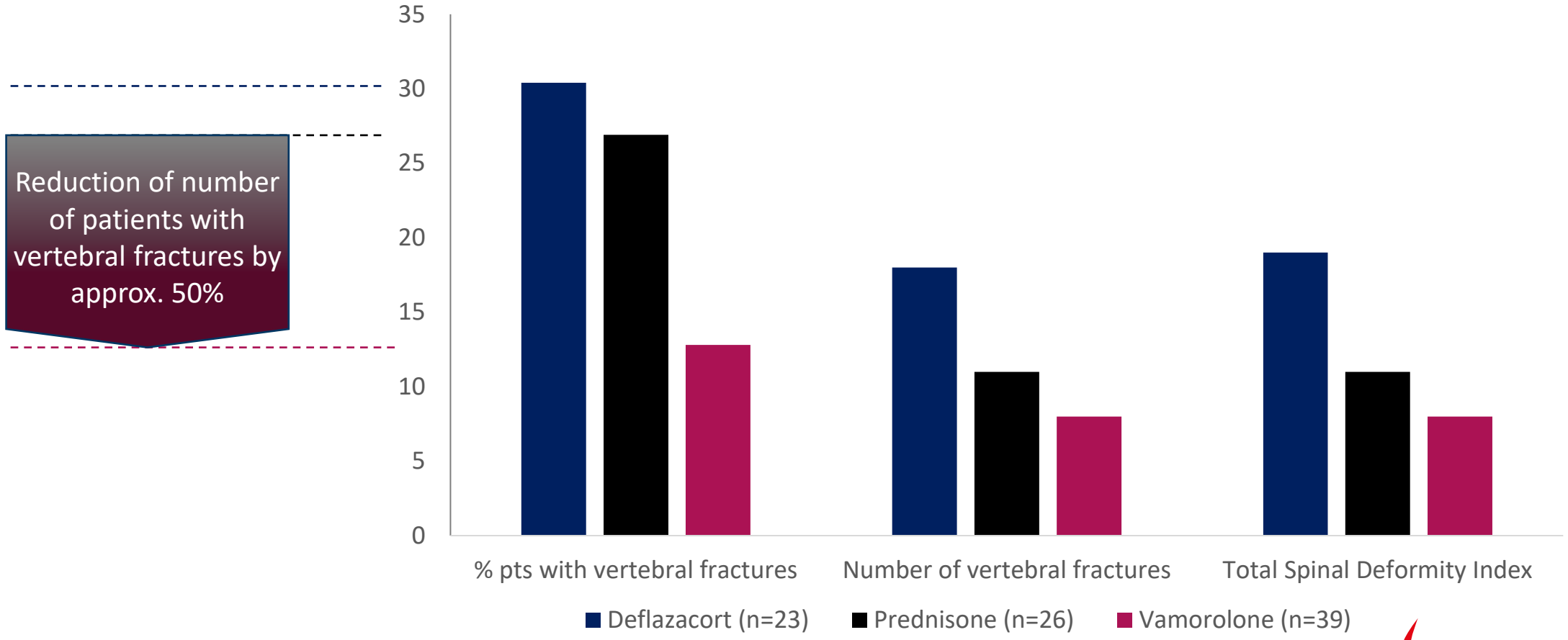
* Cross study comparisons with FOR-DMD as external control specified prior to data base lock in the statistical analysis plan of the VISION-DMD pivotal study.

santhera
THEIR FUTURE - OUR FOCUS

Fewer and less severe spinal fractures with vamorolone compared to classical corticosteroids over 2.5 years

Bone Health

Vamorolone long-term extension (LTE) study vs FOR-DMD, matched comparison, central reading using modified Genant grades[1]



1: https://www.santhera.com/assets/files/content/scientific-literature/FP03-WMS_poster_20_August_2022.pdf
Spinal Deformity Index (SDI): sum of the Genant Grades from T4 to L4, and therefore, is the composite of both fracture number and severity

santhera THEIR FUTURE - OUR FOCUS

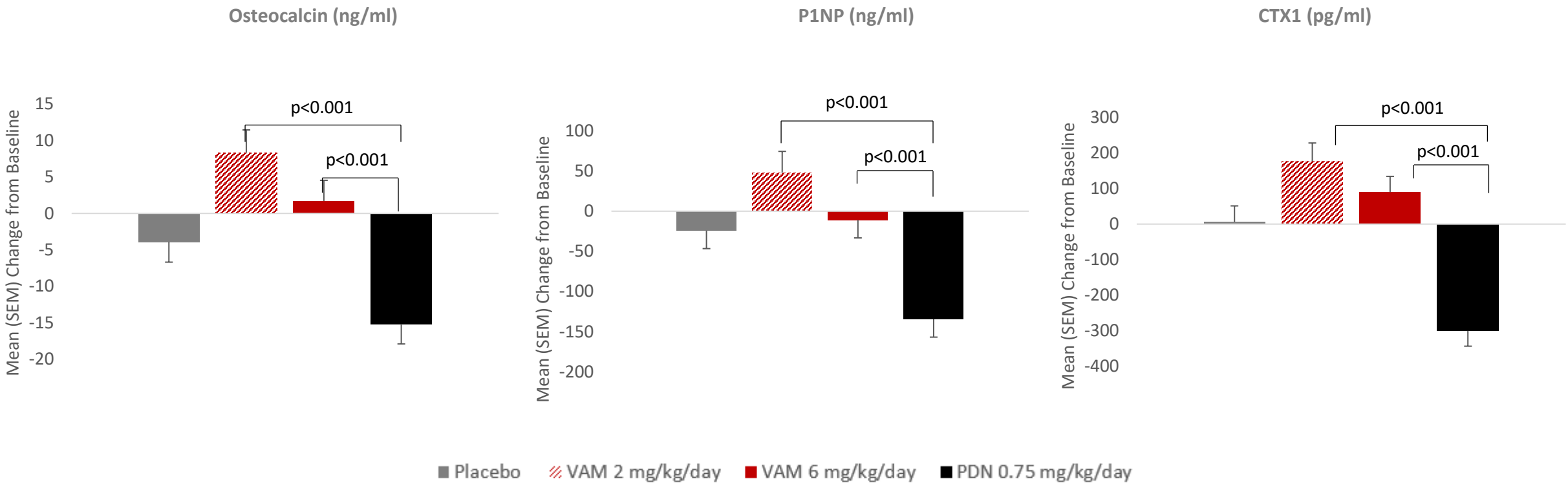
Bone biomarker data from VISION-DMD study supports findings on long-term bone health

Unlike classical corticosteroids, vamorolone does not have a negative impact

Bone Health

Biomarkers of bone formation[1]

Biomarkers of bone remodelling[1]



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

santhera THEIR FUTURE - OUR FOCUS

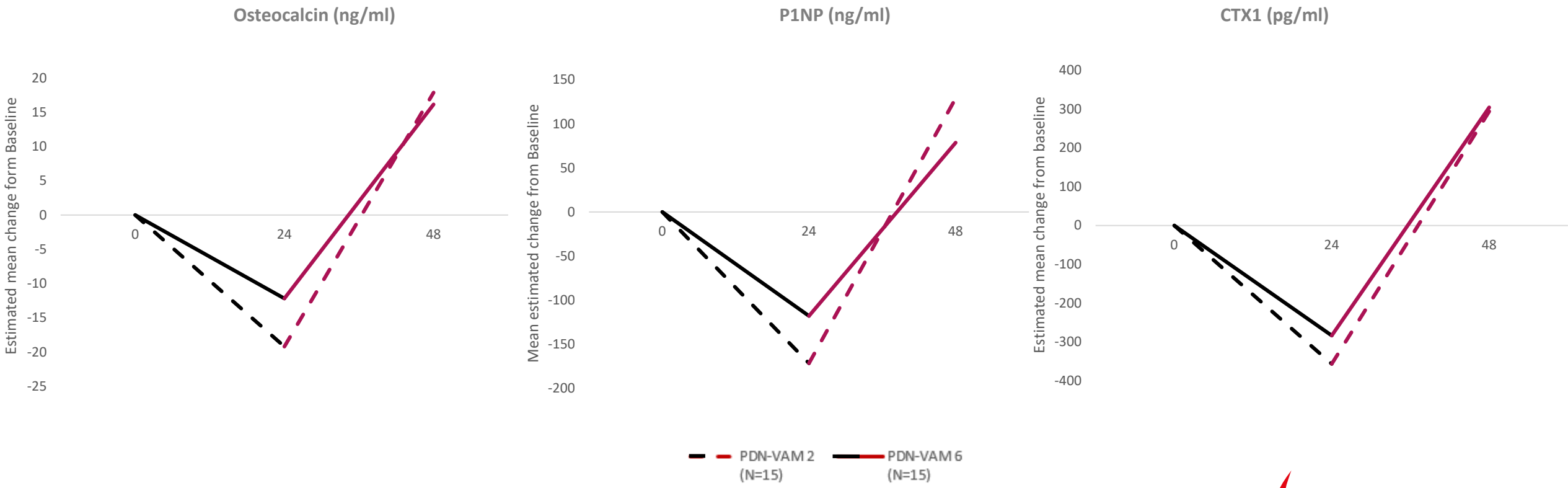
Bone biomarker data from VISION-DMD study supports findings on long-term bone health

Rapid recovery of bone biomarkers after switching from prednisone

Bone Health

Biomarkers of bone formation[1]

Biomarkers of bone remodelling[1]



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

santhera
THEIR FUTURE - OUR FOCUS

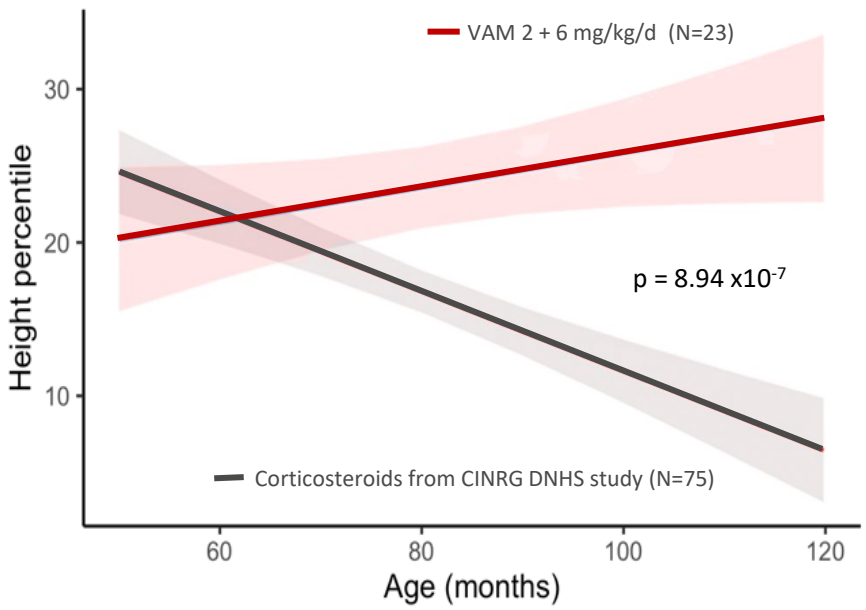
Vamorolone allows for normal bone development and growth

Comparison to natural history data and in patients switching from prednisone

Bone Health

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 Data[2]



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

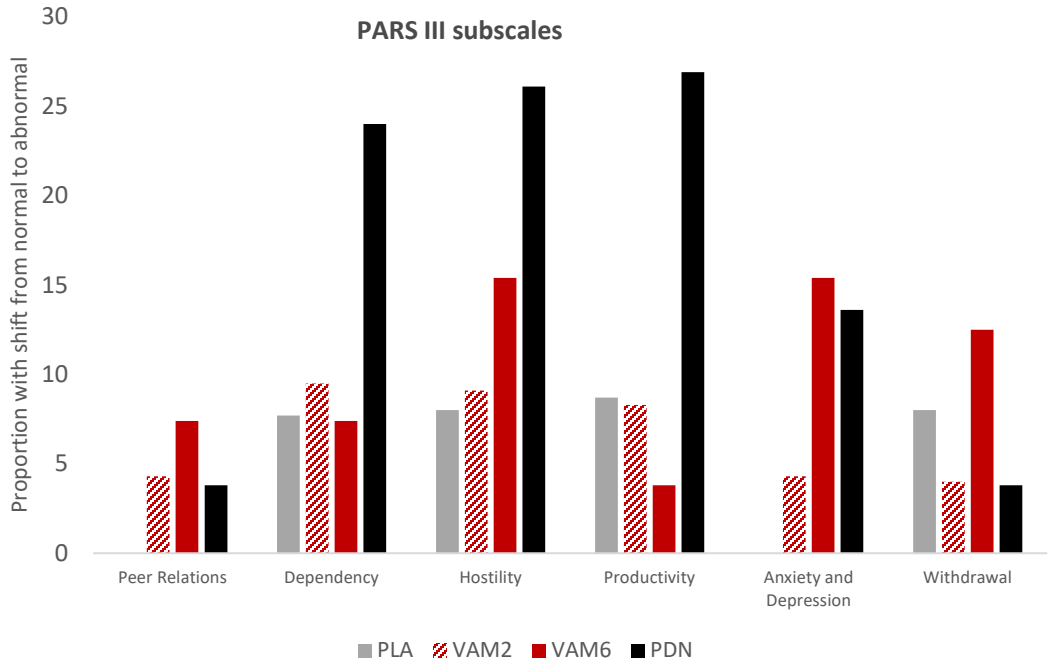
1. Safety Population 2 (SAF-2); PDN – Prednisone 0.75 mg/kg/d; PDN-VAM: growth trajectory (z-score) compared for prednisone in Period 1 and vamorolone (2 + 6 mg/kg/d) in Period 2; All doses daily; MMRM estimates of changes from baseline 2. Mah et al; ePoster WMS 2021

santhera THEIR FUTURE - OUR FOCUS

Fewer and less severe behavioral problems reported with vamorolone

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

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



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

VISION-DMD Study Data on File Santhera 2022

santhera
THEIR FUTURE - OUR FOCUS

AGAMREE® (vamorolone) clinical data value proposition

- **Durable efficacy comparable to standard of care with AGAMREE® 6 mg/kg/day**
  - Statistically robust efficacy vs placebo at 24 weeks for both 2 mg/kg/day and 6 mg/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 corticosteroids at 48 weeks

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

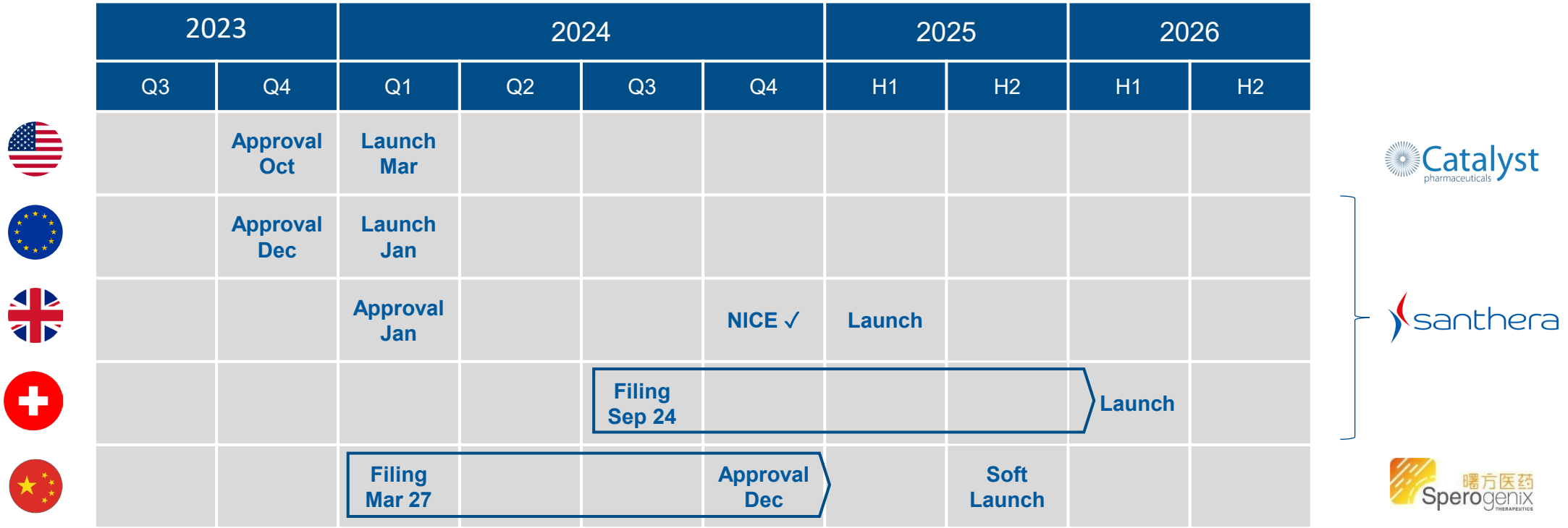
- **Improved safety profile compared to prednisone evident in the first 24 weeks**
  - Placebo-like treatment emergent adverse events (TEAEs) with vamorolone 2 mg/kg/day
  - Fewer and milder TEAEs with vamorolone 6mg/kg/day compared to prednisone, including behavioral problems

- **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**

santhera
THEIR FUTURE – OUR FOCUS

Full approval by FDA, EMA, MHRA and NMPA for AGAMREE® in DMD

- Regulatory filings under review in Switzerland and Israel
- Orphan drug exclusivity in U.S. (7 years) and Europe (12 years incl. pediatric extension)
- Patent protection at least until 2040 (U.S.) and 2035 (EU)



US: Launch by partner Catalyst
EU: Staggered launch by Santhera with first country Germany started in January 2024
UK: Positive recommendation by NICE (National Institute for Health and Care Excellence)
CH: Outcome expected in late H1-2026 or early 2026 if Article 13 (including foreign authorities` assessment) is accepted
CN: Approved by NMPA and DO in HK in Dec 2024; Soft launch while under pricing negotiations

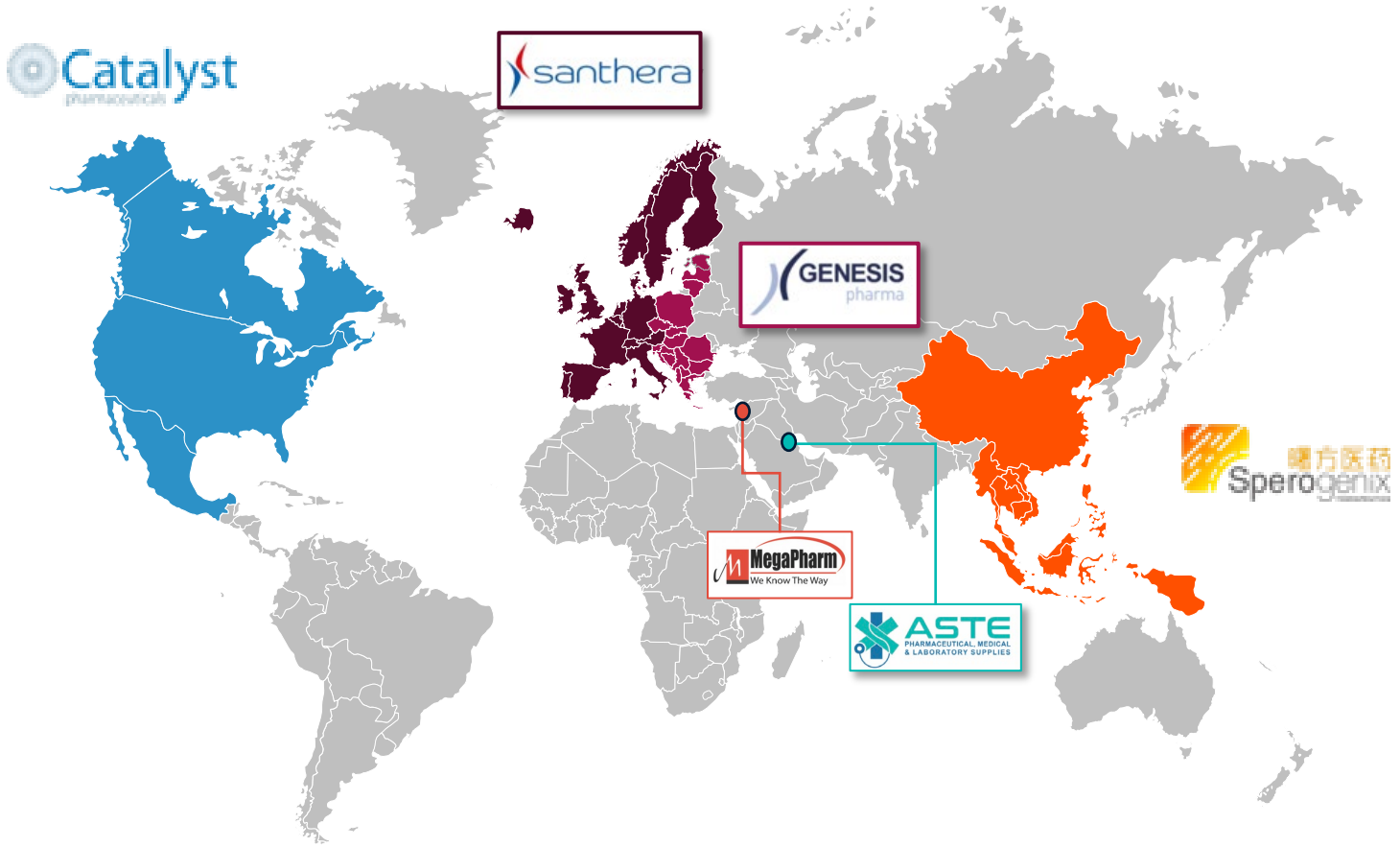
santhera THEIR FUTURE - OUR FOCUS

Stepwise global expansion with strong partners under way

Santhera holds global rights in all territories not yet covered by agreements

Geo-expansion

- Santhera is actively pursuing further international partnerships
- Currently patients are treated with Agamree in the US, multiple European countries, Israel, China and Qatar

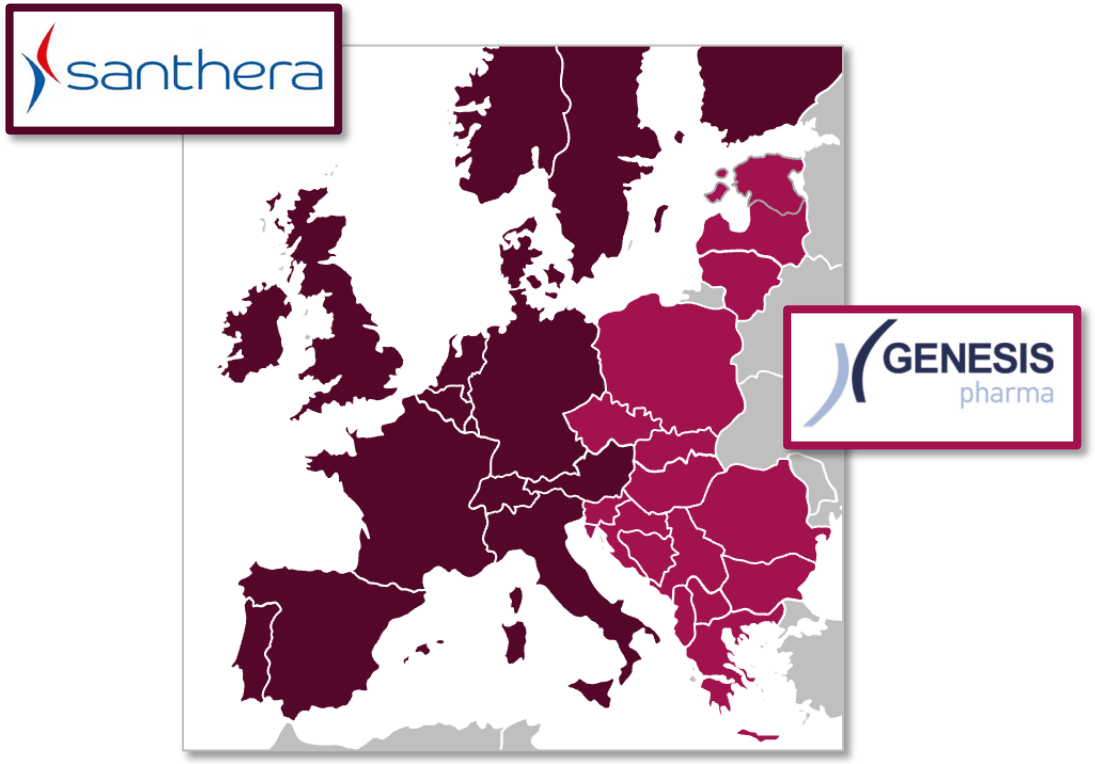


santhera
THEIR FUTURE - OUR FOCUS

Santhera commercial launch in key European geographies

Santhera aims to market vamorolone in DMD itself in territory with population of ~ 410 million

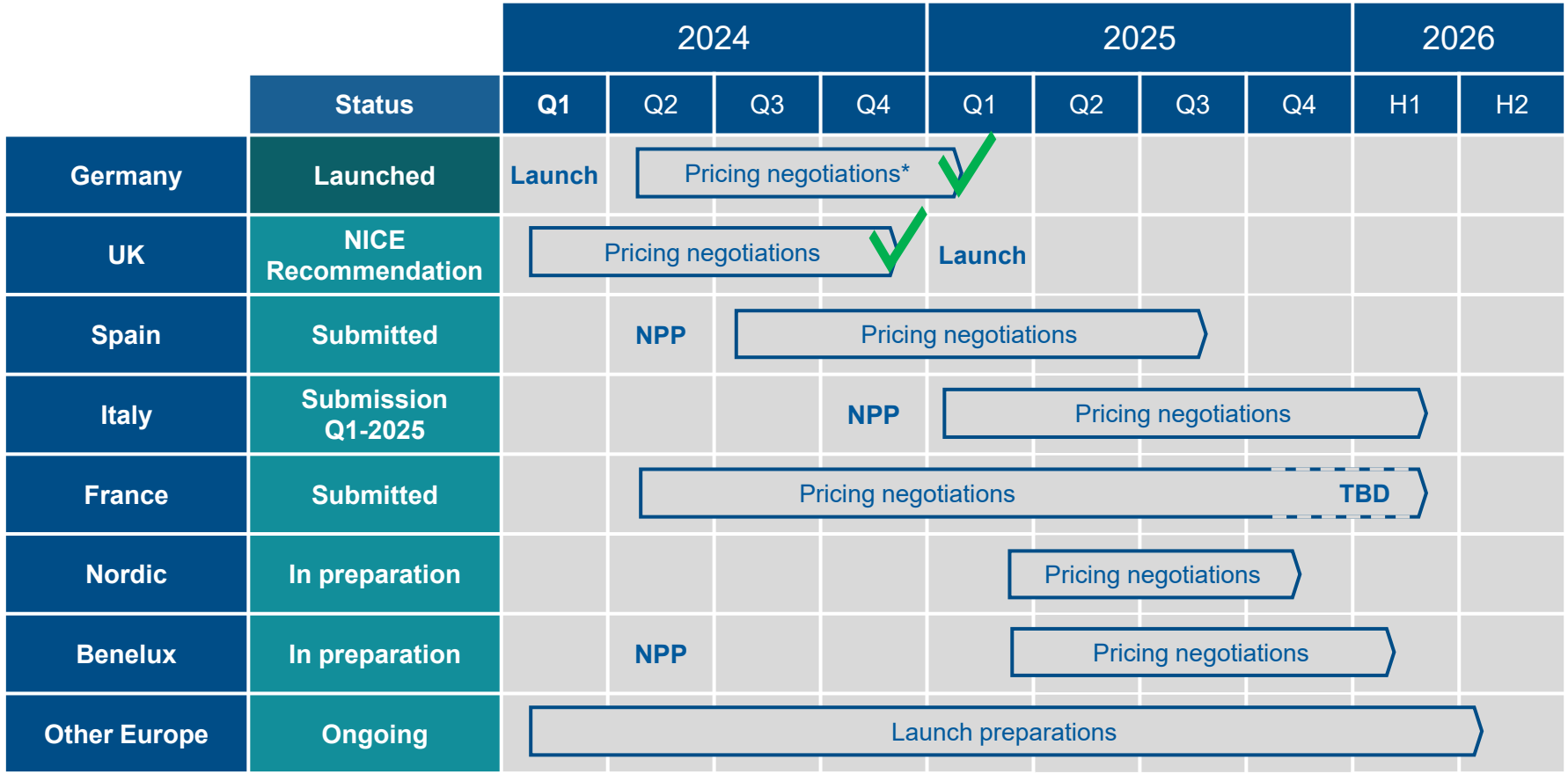
- **First launch in Germany in January 2024**
  - Staged roll-out across the key European markets
  - Strong and growing stakeholder support
- **Lean commercial organization**
  - Up to 50 incremental employees over next two years
  - Country activities supported by central hub
- **European market opportunity in DMD alone**
  - Expected peak sales of EUR >150 million in Santhera territory
  - Additional revenue from distribution partner Genesis



santhera
THEIR FUTURE - OUR FOCUS

Market access and launch milestones in Europe

- **UK in Q1-2025 will follow Germany as the second country with commercial, reimbursed launch**
- **Several other countries are in market access discussions with authorities with launches planned for 2025**

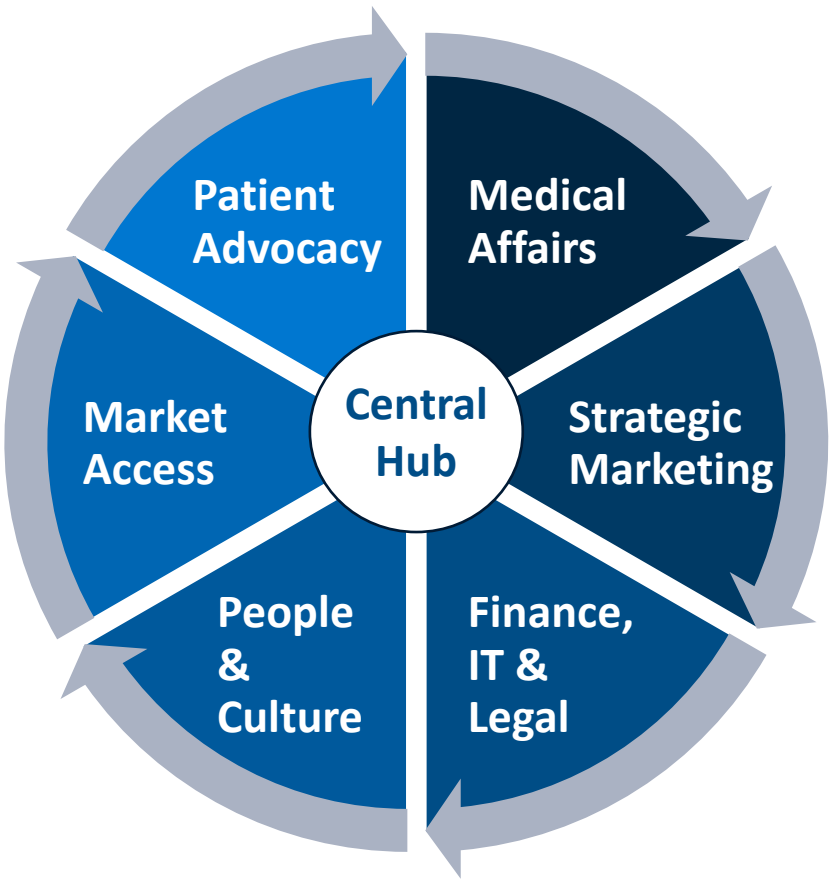


* Decision expect Mid-January 2025; NPP: Named Patient Program; NICE: National Institute for Health and Care Excellence

santhera
THEIR FUTURE – OUR FOCUS

Santhera commercial set-up with central hub structure at headquarters

Headquarter core functions collaborate with license partners and support own lean country teams as well as distribution partners



License Partners
- Catalyst
- Sperogenix

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

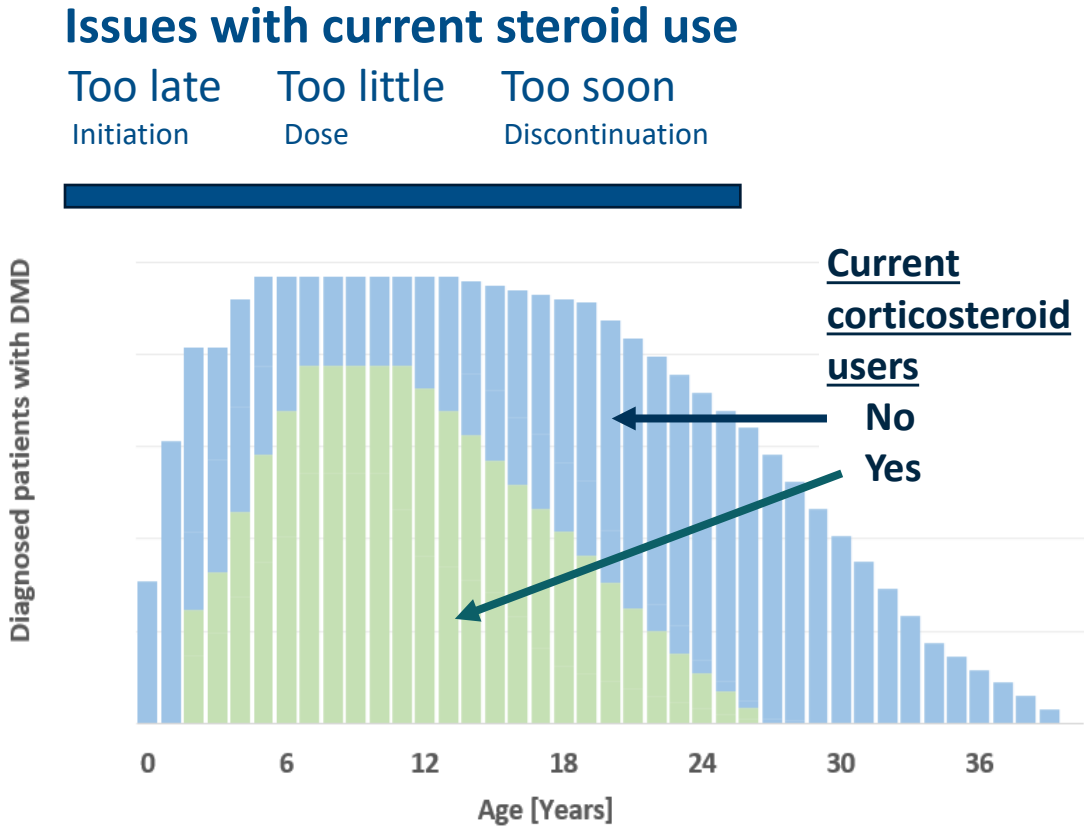
Distributors
- Genesis (Selected European countries)
- Others

Benelux: Belgium, Netherlands, Luxembourg; Nordics: Denmark, Sweden, Norway, Finland

Market opportunity to change the foundational therapy in DMD

AGAMREE® can adress the shortcomings of current standard of care corticosteroid use

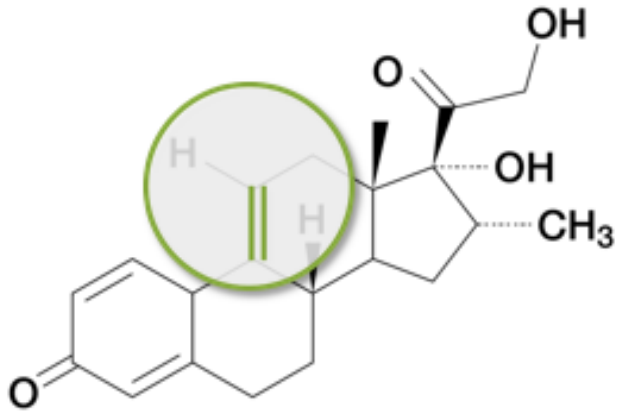
- **AGAMREE ® opportunity for change**
  - Replacing current corticosteroid treatment initiation
  - Switching patients from standard corticosteroids
  - Restarting treatment for patients recently discontinued
- **Market Size**
  - In Santhera´s own commercialized markets, there are about 13,000 patients with DMD, of which approximately 8,000 boys/men are being treated with standard corticosteroids[1,2]
- **AGAMREE® potential**
  - In own markets, up to 5,000 patients are expected to be on AGAMREE® by end of 2030
  - In own markets, standard range of orphan drug pricing leads to a peak sales estimate of EUR >150 million
  - Global sales of CHF >150 million are expected by 2028 already (including royalties)



1: Cowen et al., 2019, BMC Neurology; 2: Ryder et al., 2017, Orphanet Journal Rare Diseases

santhera
THEIR FUTURE - OUR FOCUS

Vamorolone in Becker muscular dystrophy



Becker muscular dystrophy (BMD) disease profile and corticosteroid use

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

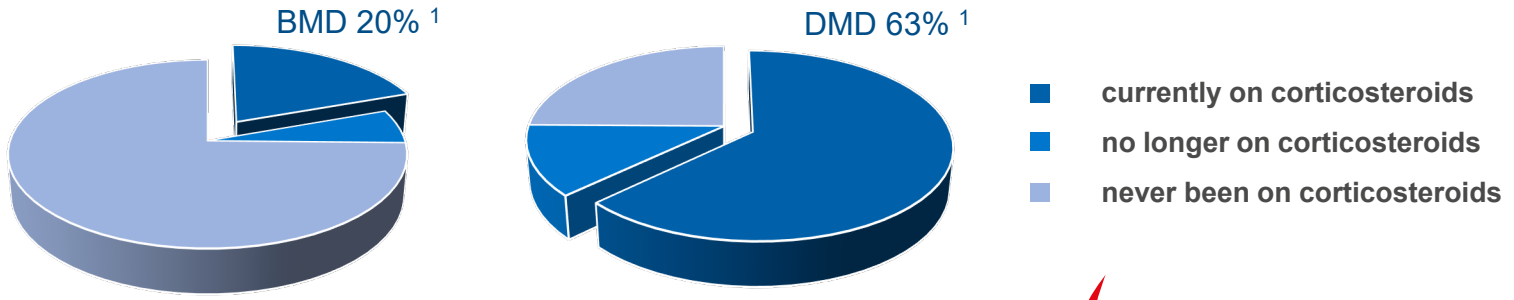
CORTICOSTEROIDS IN BMD

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



1 Cowen et al., 2019, BMC Neurology

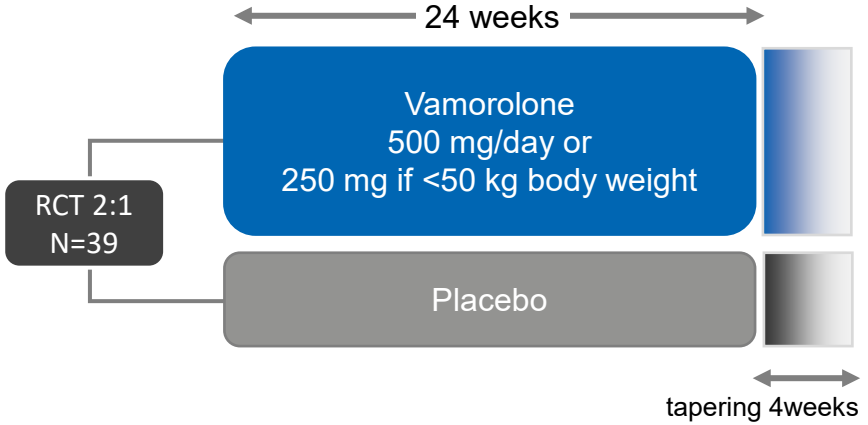
santhera
THEIR FUTURE - OUR FOCUS

Vamorolone holds promise in BMD based on data generated for DMD

Vamorolone designated orphan drug status by FDA in January 2024

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



Phase 2 trial of **vamorolone** in steroid-naive patients aged 18 – 64 years

	NCT05166109
Sponsor	ReveraGen
Objectives	Safety and efficacy
Centers	Pittsburgh (USA), Padua (IT)
PI	P. Clemens, USA
Funding	FDA , NIH, Foundation Eradicate Duchenne

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

santhera
THEIR FUTURE - OUR FOCUS

Trading update (March 4, 2025)

santhera
THEIR FUTURE - OUR FOCUS

2024

Full Year Trading update

Trading update for the fiscal year 2024 and outlook for 2025 and beyond

- **Total revenue** from contracts with customers: **CHF 39.1 million** (2023 CHF 103.4 million), driven by strong underlying revenue growth offset by significant licensing milestones recognised in 2023 from out-licensing activities in major territories
- **Product sales**: **CHF 14.8 million** (2023 CHF 0.8 million) driven by the successful launch of AGAMREE in Germany and Austria.
- **Royalties & milestones**: **CHF 19.3** million (2023 CHF 99.9 million), 2023 revenues were bolstered by out-licensing milestones received from Catalyst Pharmaceuticals in the U.S. and Sperogenix in China.
- **Revenue from supply of product and services to partners**: **CHF 5.0** million (2023 CHF 2.7 million)
- **Cash and cash equivalents**: **CHF 41.0** million (2023 CHF 30.3 million).
- **Cash runway**: **Extended to mid 2026** at which point the Company expects to be **cash breakeven**.

https://www.santhera.com/assets/files/financial_reports/2023_SANN_AnnualReport2023_final.pdf
santhera
THEIR FUTURE - OUR FOCUS

Santhera financial status

Santhera Pharmaceuticals is listed on the Swiss Stock Exchange SIX: Ticker SANN

- **Cash runway**
  - December 2024: CHF 41 million
  - Financing extends runway into 2026 and planned cash breakeven

- **Key figures prior to financing Aug 2024**
  (CHF million as of June 30, 2024 / 6-month period)*

Net revenue	14.1
Net (loss)	(15.3)
Cash (used) in operations	(15.3)
Cash & cash equivalents	16.5
Convertible bonds (maturity August 2024)	(23.7)
Shareholders' equity	48.1

- **Recent milestones AGAMREE® for DMD**
  - ✓ 01-2024: Launch in Germany on Jan 15, 2024
  - ✓ 03-2024: Launch in U.S. on Mar 13, 2024
  - ✓ 08-2024: Up to CHF 69 million financing extending runway
  - ✓ 09-2024: Distribution partner Genesis (Selected European)
  - ✓ 09-2024: MAA accepted for review by Swissmedic
  - ✓ 12-2024: Approval in China/Hong Kong for partner Sperogenix
  - ✓ 01-2025: Positive NICE Final Guidance
  - ✓ 02-2025: Agreement with German GKV-SV

- **Capital structure**
  - Basic shares outstanding 13.5 million
  - Market capitalization CHF 214 million (per share CHF 15.82)
  - Major shareholders Catalyst, Idorsia and Highbridge Capital
  - Research by H.C. Wainwright, Octavian and ValuationLAB

*All amounts consolidated IFRS and unaudited

santhera
THEIR FUTURE - OUR FOCUS

Santhera Pharmaceuticals

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

March 2025