# Santhera Pharmaceuticals

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

Corporate Presentation

October 1, 2024

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# 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 EU 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 three authorities (US, EU, UK)

FDA (10/2023), EMA/EC (12/2023) and MHRA (01/2024)

Launched in Germany by Santhera and U.S. by partner Catalyst

NDA filed in China and priority review granted to partner Sperogenix

## Finance

New financing in August 2024

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

Major shareholders: Catalyst (11%), Idorsia (10%) and Highbridge (8%)

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# 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 January 11, 2024
- Potential as alternative to corticosteroids in broad range of therapeutic areas
- Own commercialization Western European countries
- Commercialization through partner Genesis in remaining EU
- Peak potential > EUR 150 million in DMD (Santhera estimate for own markets)
- Commercialization in the U.S. by partner Catalyst, in China by partner Sperogenix

**Worldwide rights for all indications**
**(licensing partners in North America & China)**

aGamree®
(vamorolone) oral suspension 40mg/mL




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

## Launch in Germany on January 15, 2024, by Santhera and on March 13, 2024, in the U.S. by Catalyst
## NDA filed in China and priority review granted to partner Sperogenix on March 27, 2024

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



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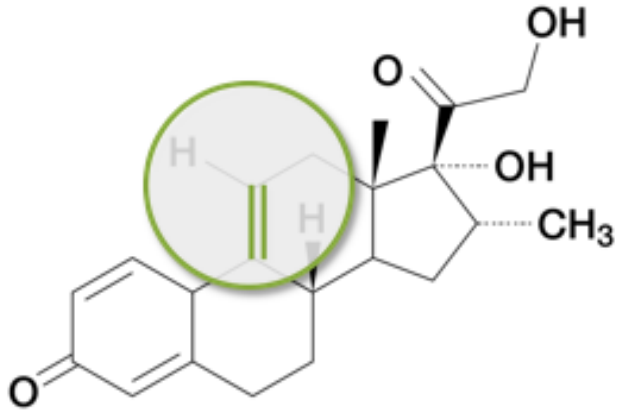
# AGAMREE® (vamorolone) in Duchenne muscular dystrophy and potentially in other disorders where current steroid use has limitations



# 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



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# 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.

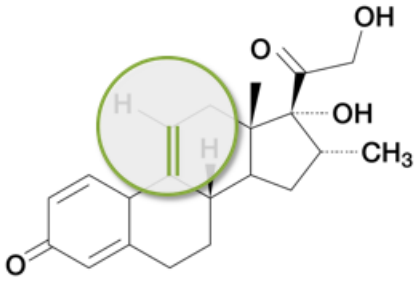
TOO LATE
TOO LITTLE
TOO SOON

### 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



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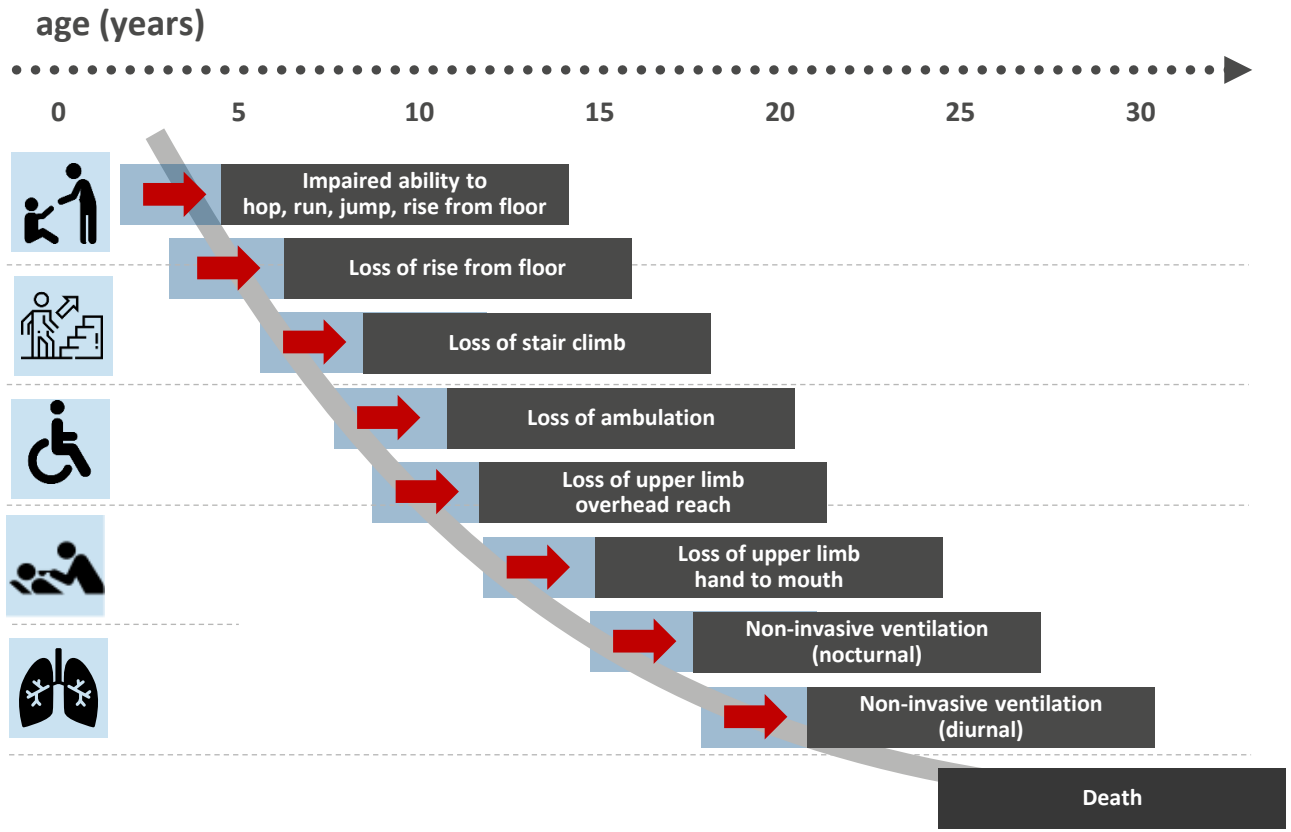
# Corticosteroids delay disease progression in DMD by 2 – 3 years[4,6]

## 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;

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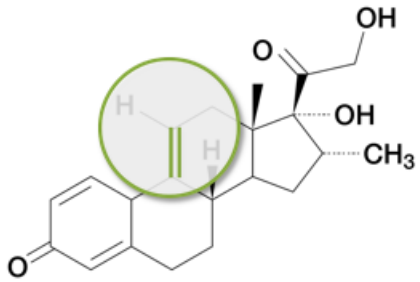
# AGAMREE® (vamorolone) dissociative properties

## 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)

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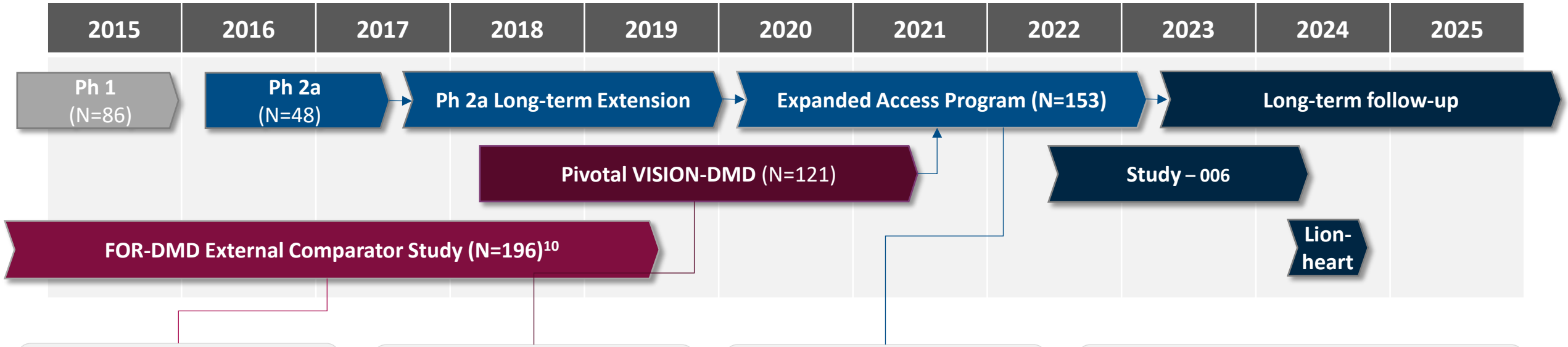
# Comprehensive AGAMREE® (vamorolone) development [2-9]

## 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

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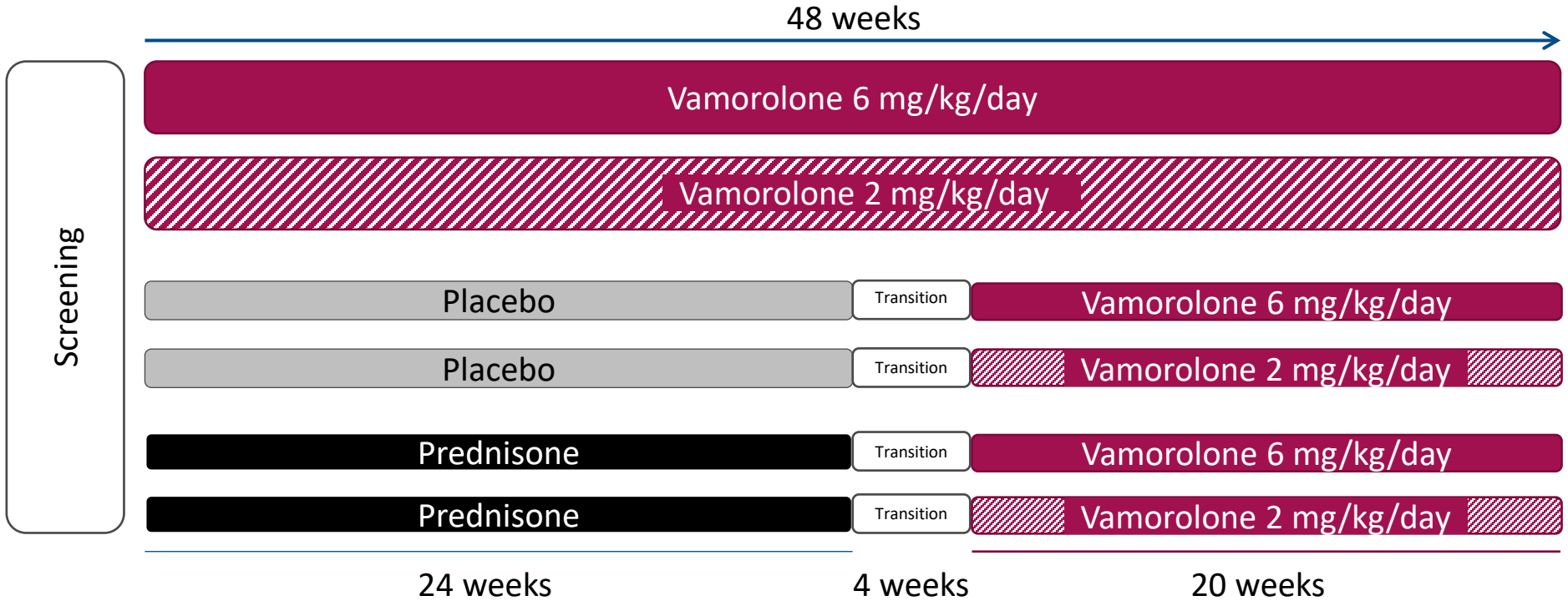
# Pivotal VISION-DMD: Study design

## 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)



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# 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



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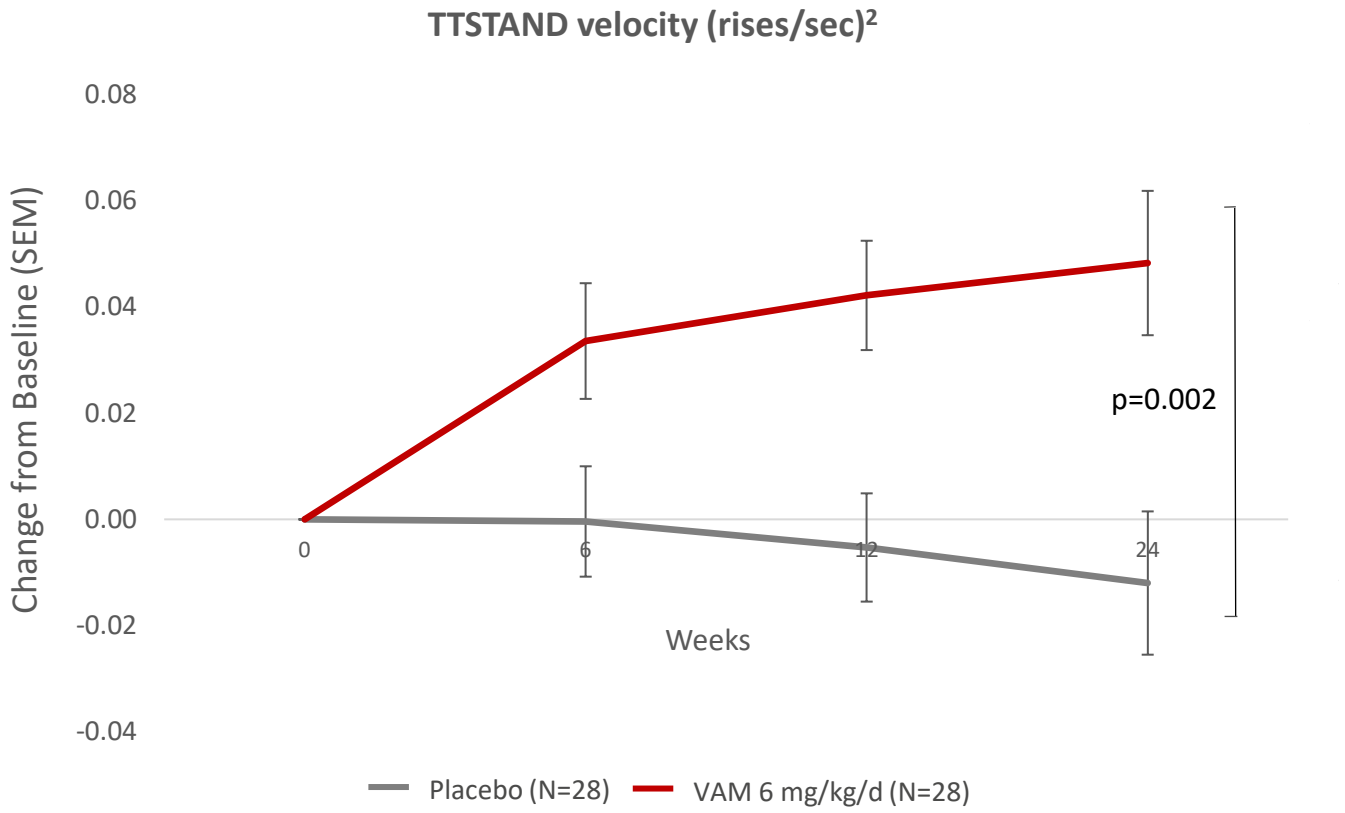
# Primary endpoint met with clinically relevant treatment difference

## 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

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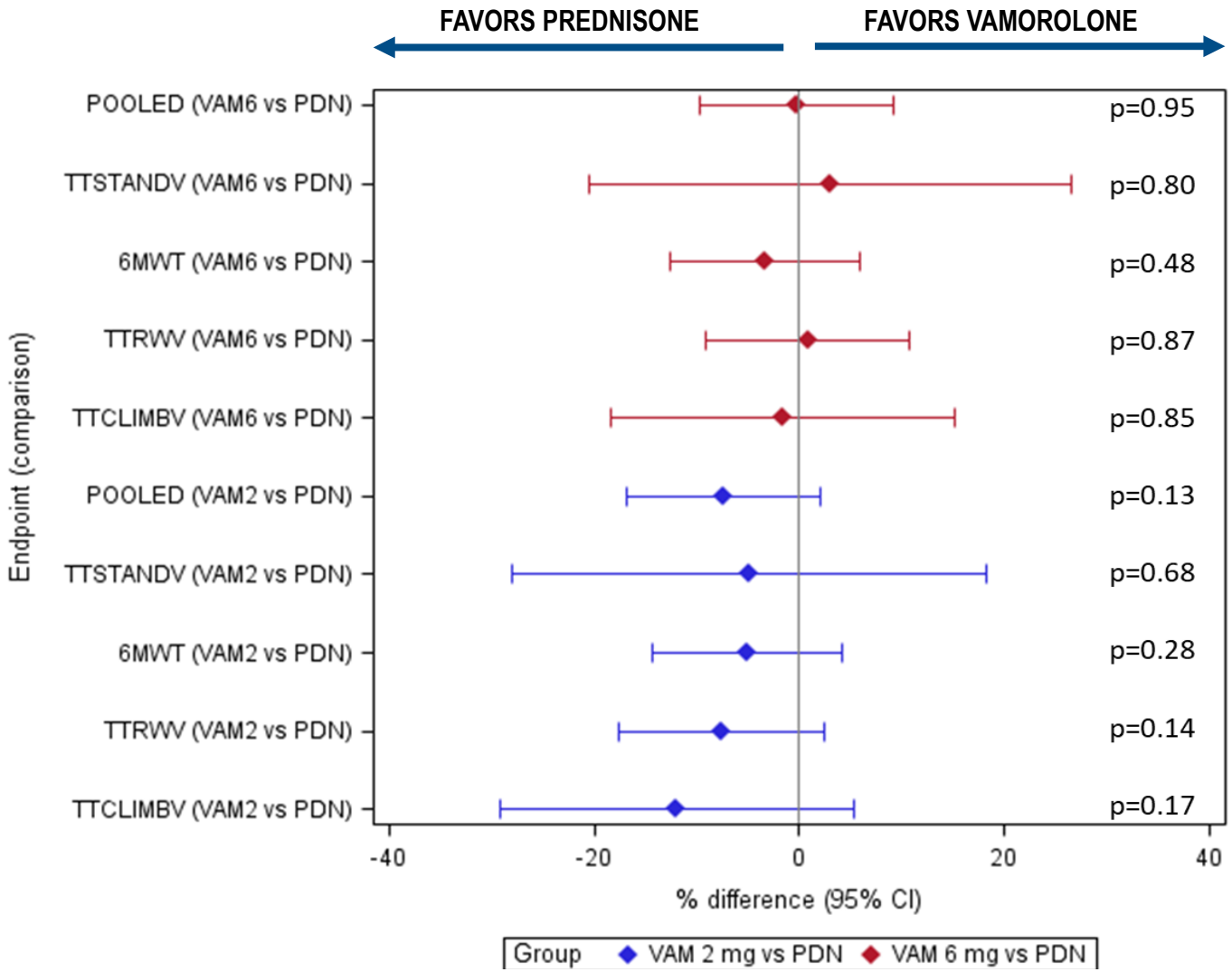
# Comparable efficacy of vamorolone 6 mg/kg/d vs prednisone 0.75 mg/kg/d

## 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



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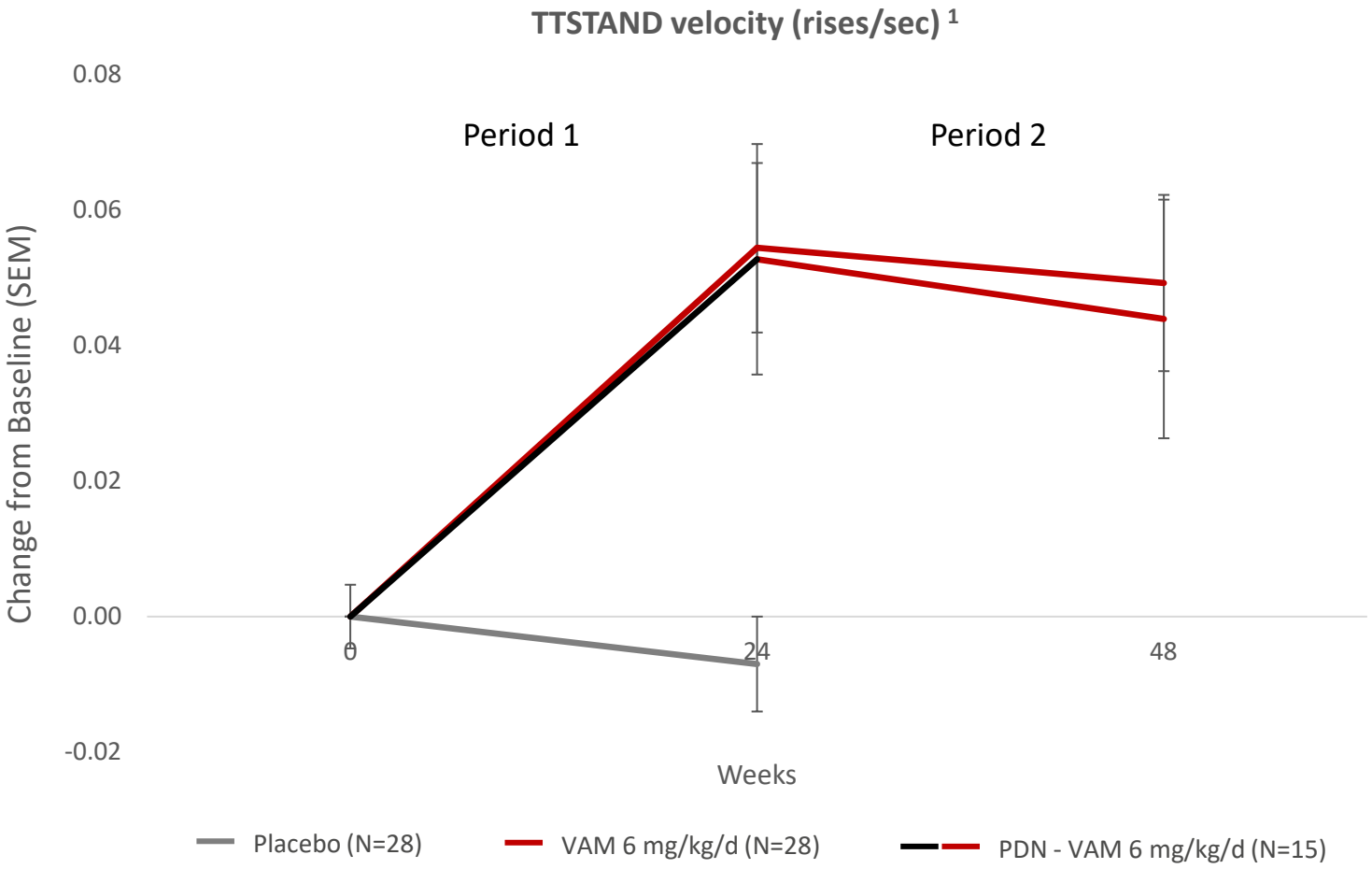
# No loss of efficacy when switching from prednisone to vamorolone

## 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]



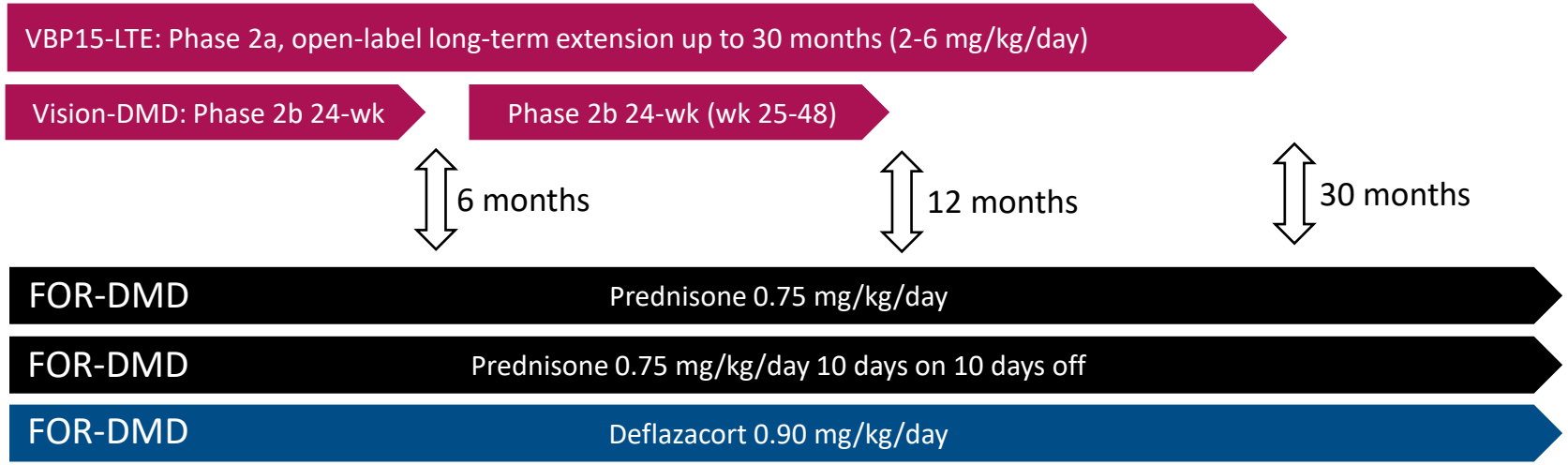
1. Data on File VAM-2021-002, mITT-2: modified intention to treat population from period 1 and 2, MMRM estimates of changes from baseline. PDN: prednisone 0.75mg/kg/day, PDN-VAM: prednisone 0.75 mg/kg/d in Period 1 transitioned to vamorolone 6mg/kg/d in Period 2 group after a 4-week tapering period;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



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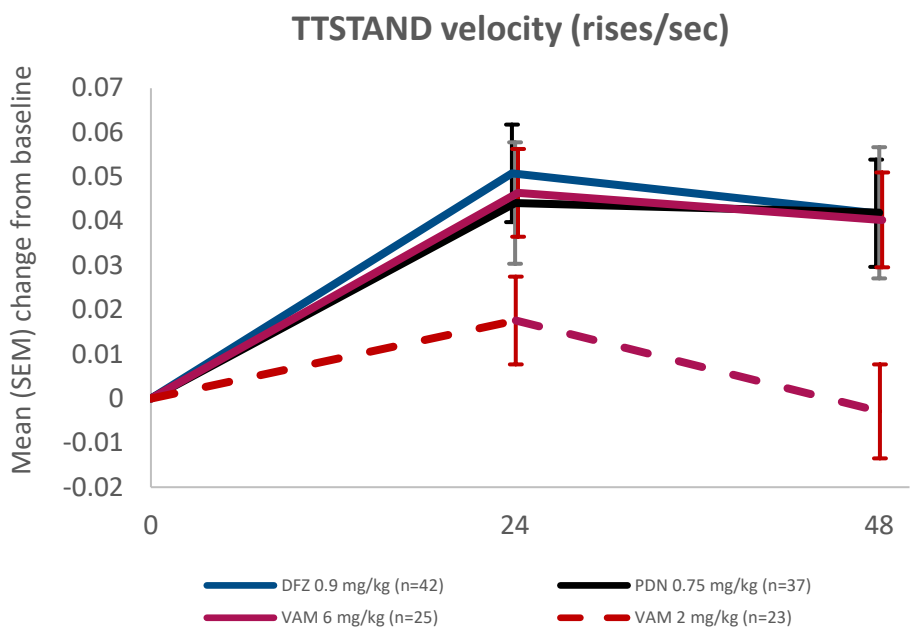
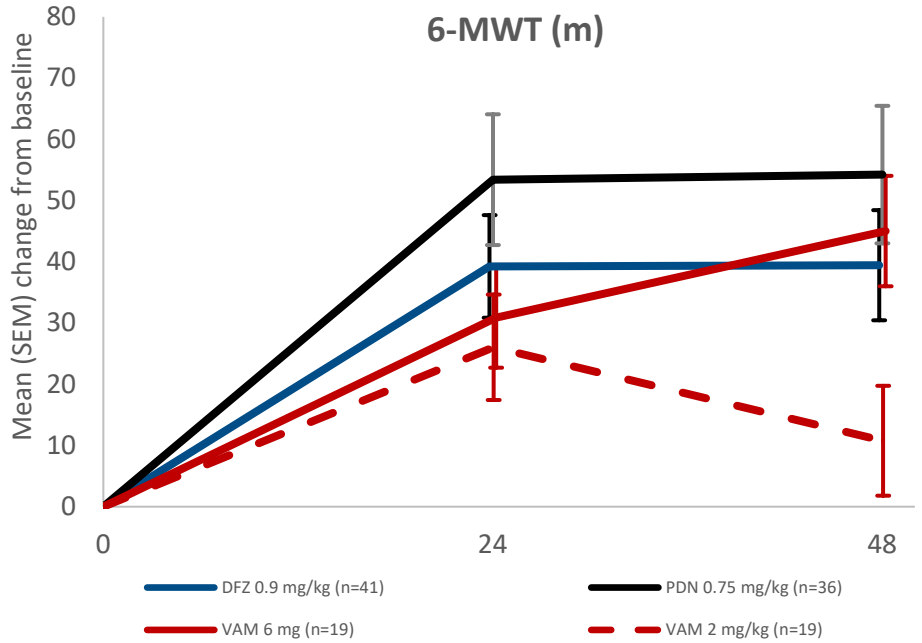
# VISION-DMD pre-specified* analyses vs FOR-DMD external control

## 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.



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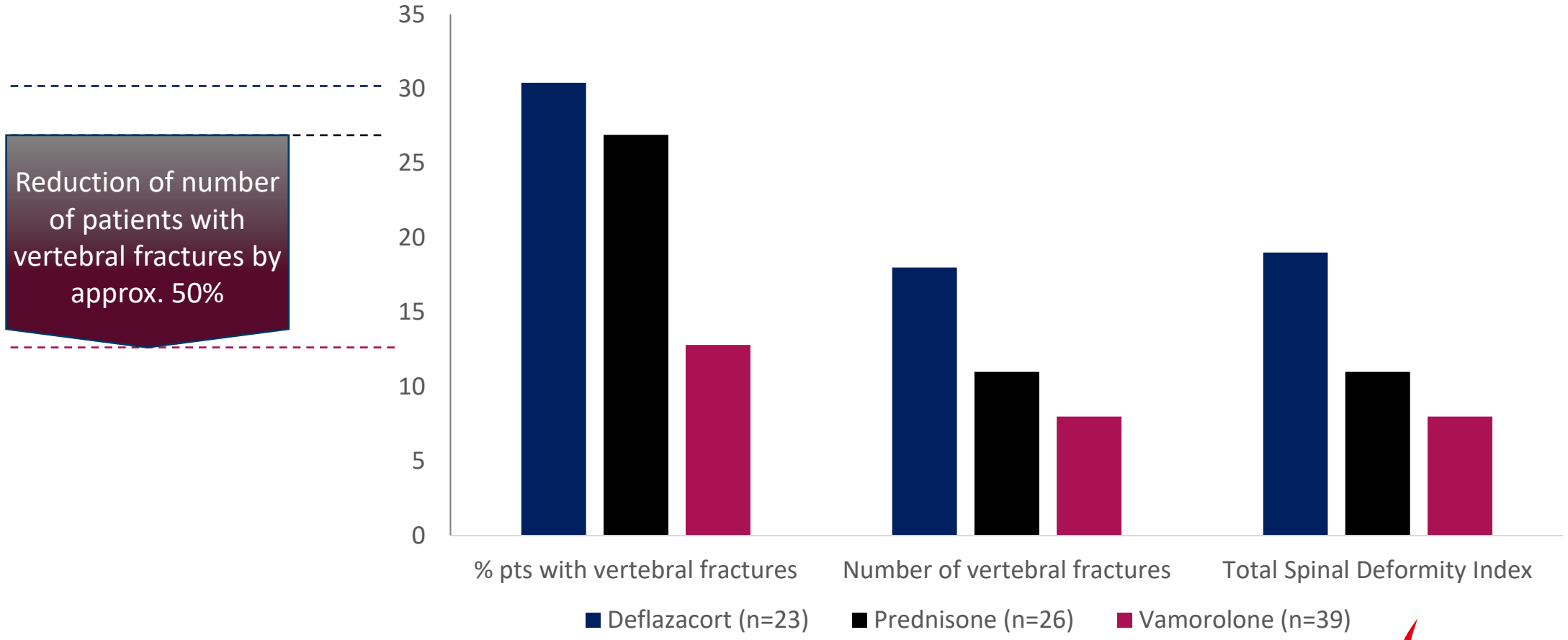
# Fewer and less severe spinal fractures with vamorolone compared to classical corticosteroids over 2.5 years

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

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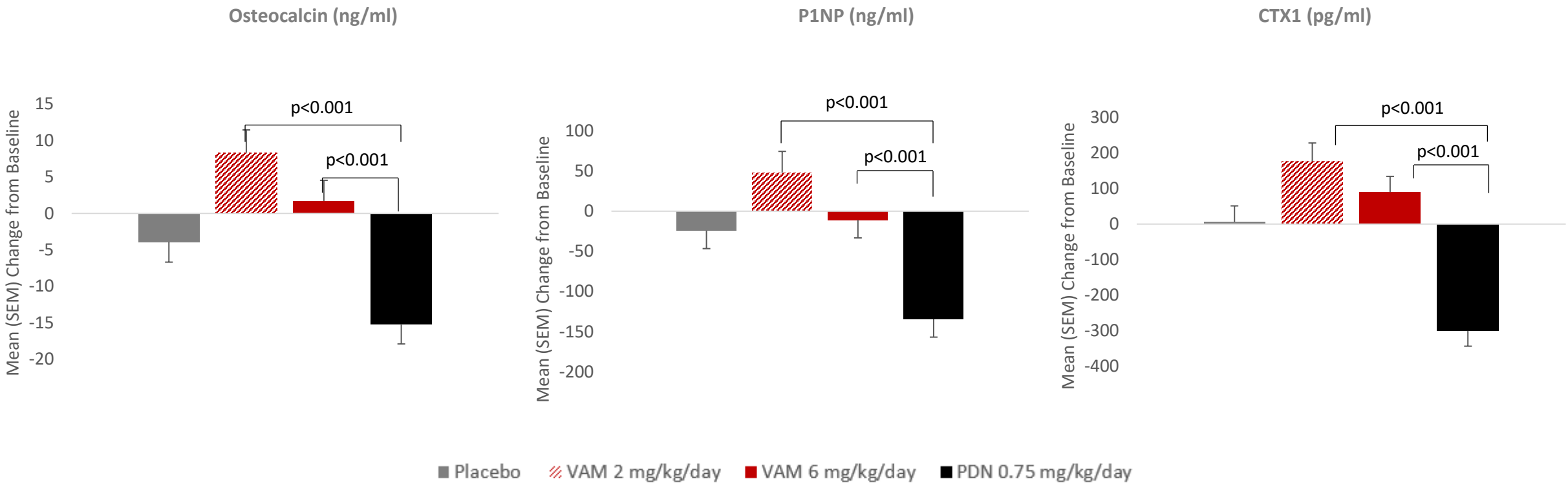
# Bone biomarker data from VISION-DMD study supports findings on long-term bone health

## 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



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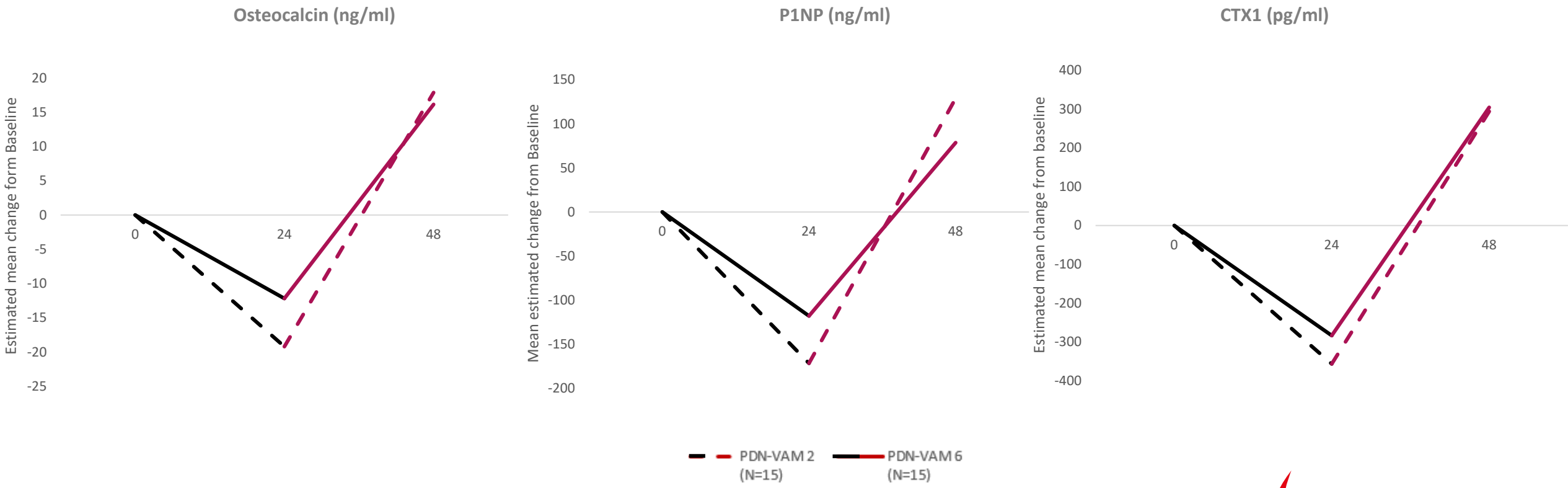
# Bone biomarker data from VISION-DMD study supports findings on long-term bone health

## 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

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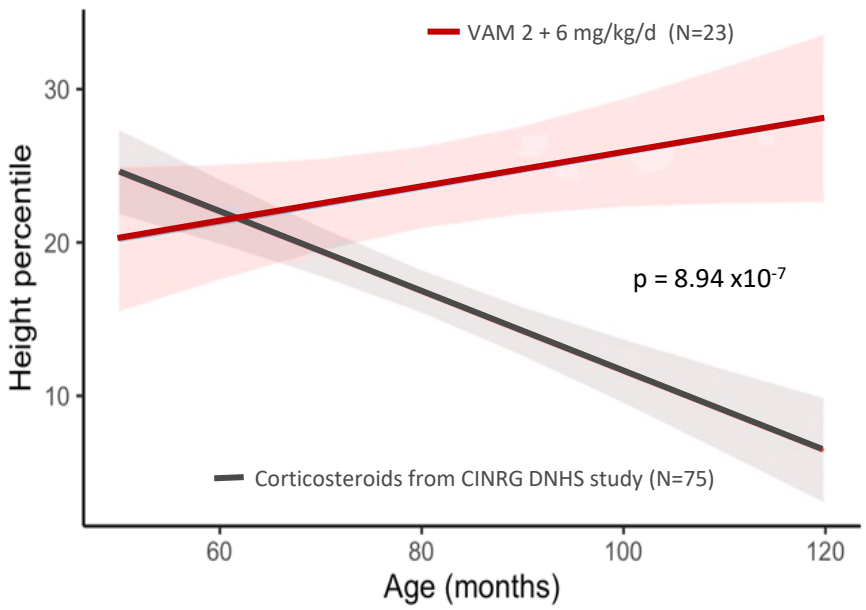
# Vamorolone allows for normal bone development and growth

## Comparison to natural history data and in patients switching from prednisone

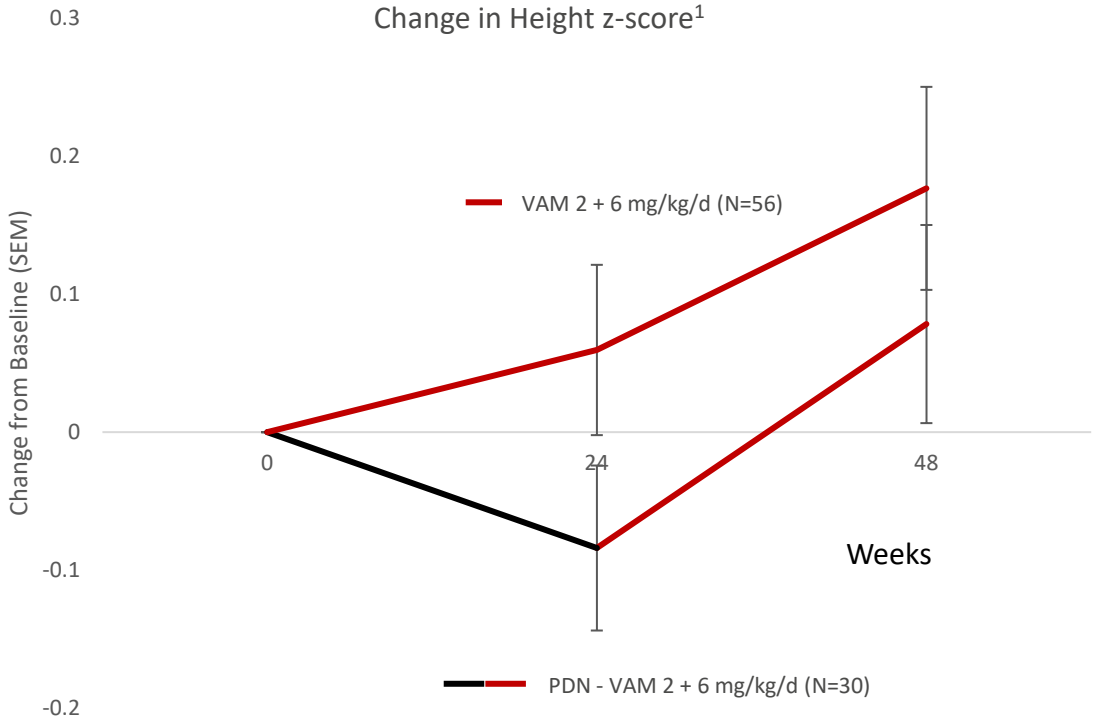
Bone Health

### 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



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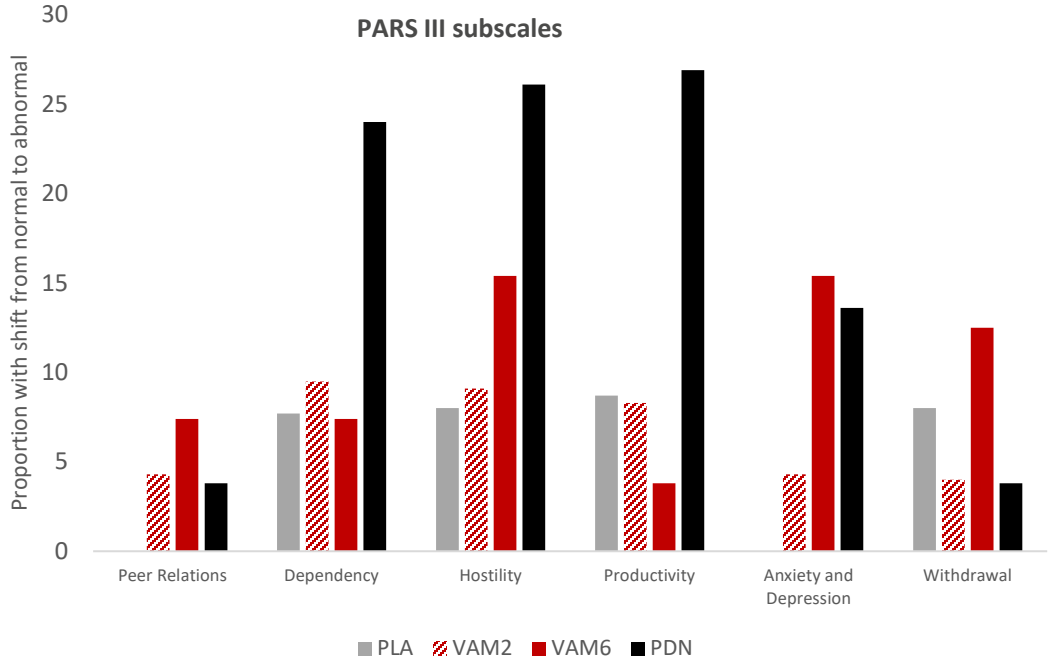
# Fewer and less severe behavioral problems reported with vamorolone

## 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

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# 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**



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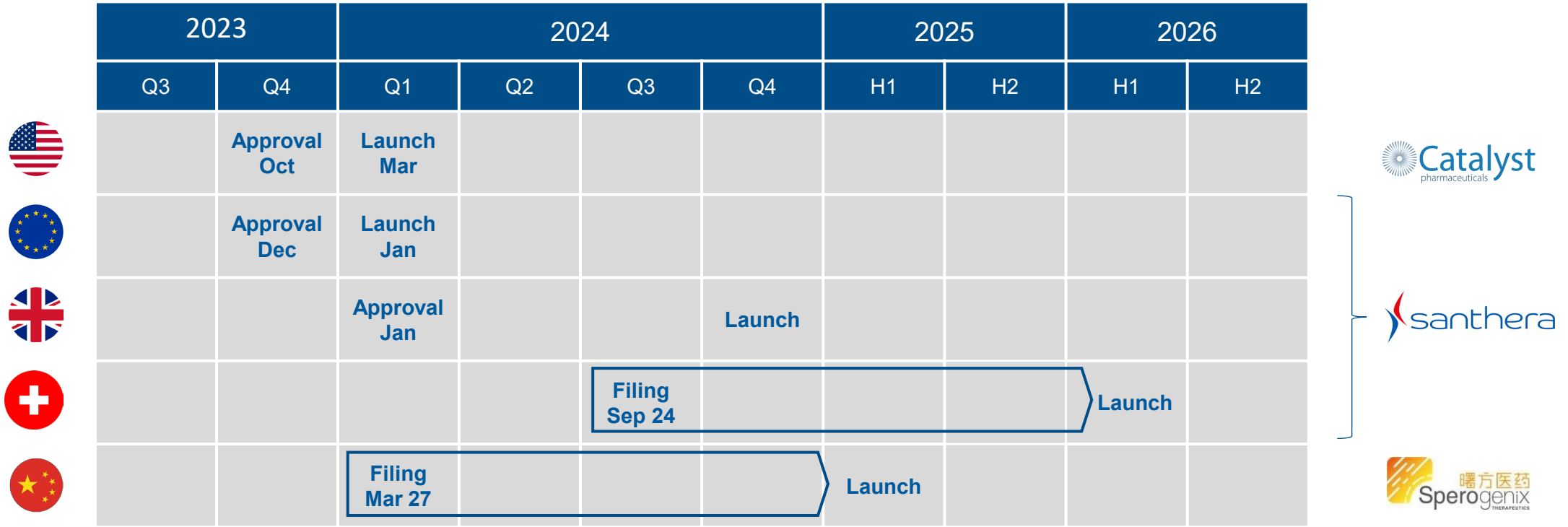
# Full approval by FDA, EMA and MHRA for AGAMREE® in DMD

- Regulatory filings under review in Switzerland and China
- 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 tarted in January 2024
UK: Pending review by NICE (National Institute for Health and Care Excellence) evaluation
CH: Outcome expected in late H1-2026 or early 2026 if Article 13 (including foreign authorities` assessment) is accepted
CN: Priority review outcome late 2024 or early 2025



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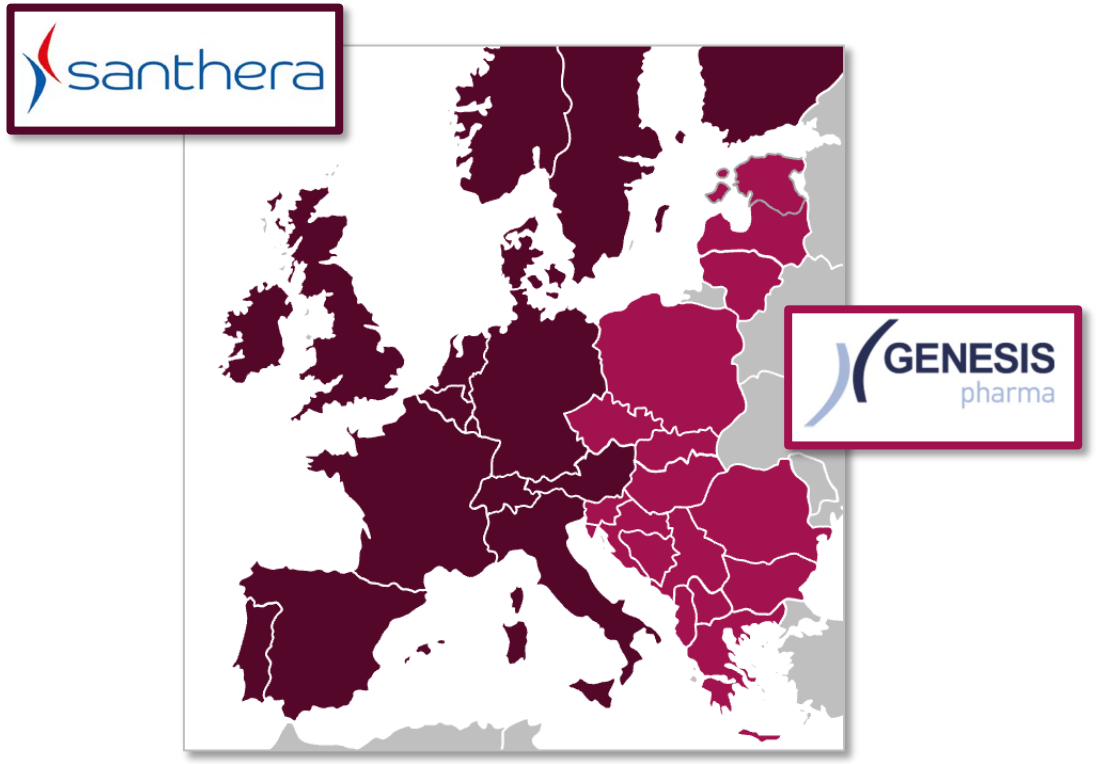
# Santhera commercial launch in key European geographies

**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



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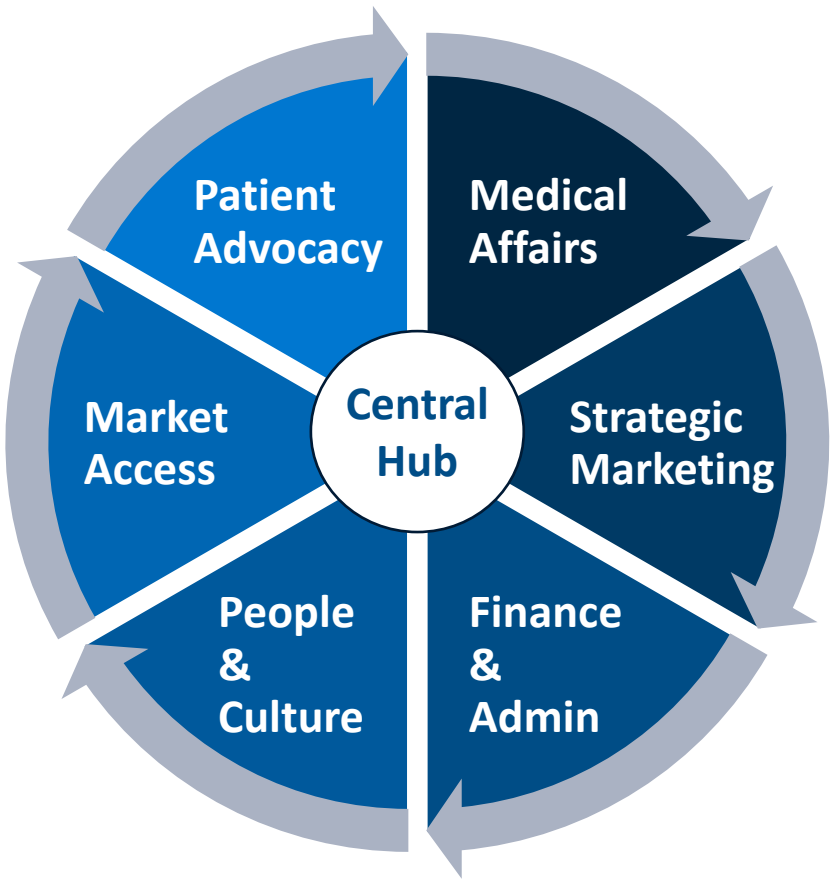
# Santhera commercial set-up with central hub structure at headquarters

**Headquarter core functions collaborate with license partners and support own lean own 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 (Eastern EU)
- RoW



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

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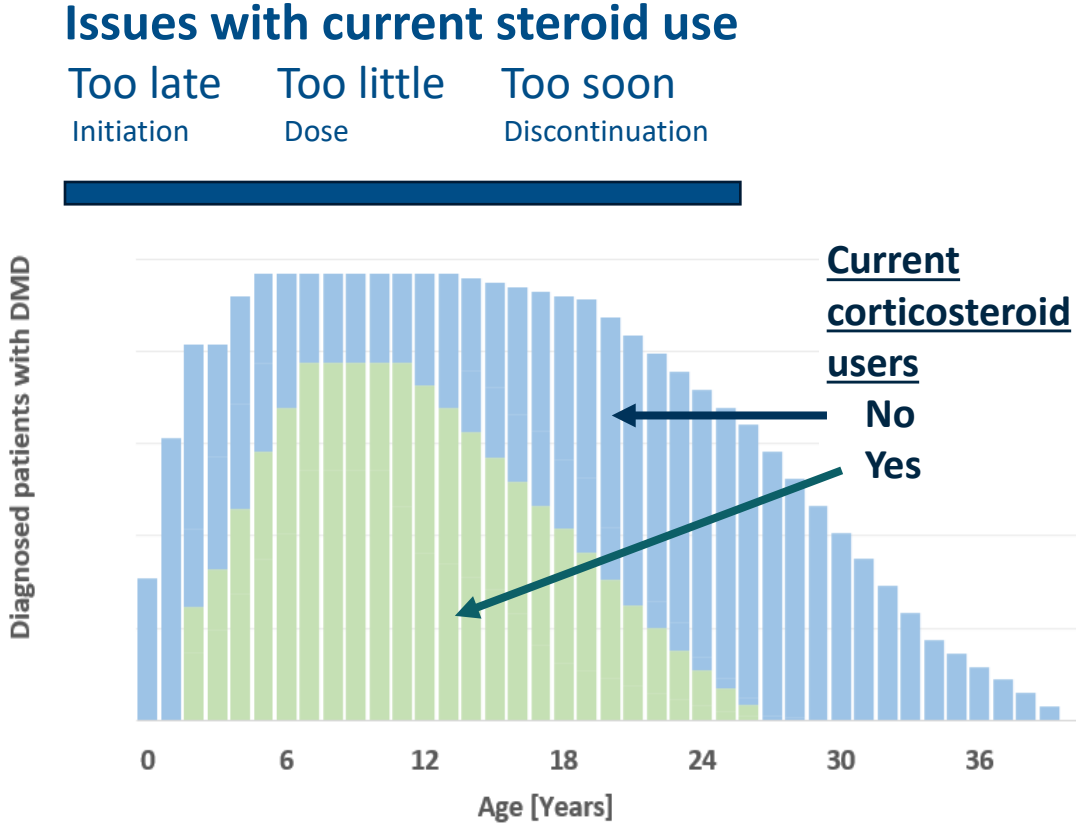
# Market opportunity to change the foundational therapy in DMD

## 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
- **Current corticosteroid use**
  - In Santhera´s own commercialized markets, there are about 12-13,000 patients with DMD, of which approximately 8,000 boys/men are being treated with standard corticosteroids[1,2]
- **Market size potential**
  - In own markets, estimated range 4,000 to 5,000 patients are expected to be on AGAMREE® by end of 2028 (50-60% share in steroid users)
  - In own markets, standard range of orphan drug pricing leads to a peak sales estimate of EUR >150 million
  - In entire Europe, sales of EUR >150 million are expected by 2028 already (including partners)






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

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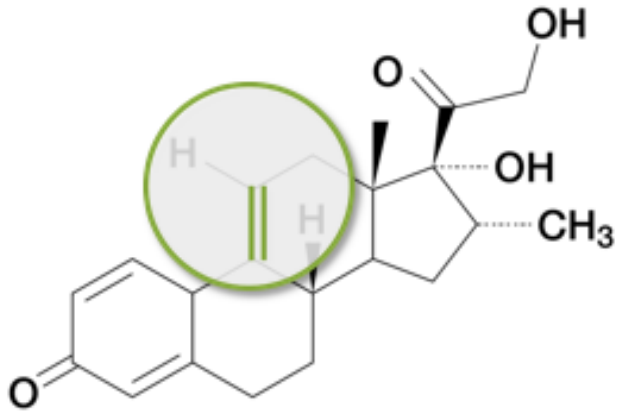
# Vamorolone in Becker muscular dystrophy



# 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 |

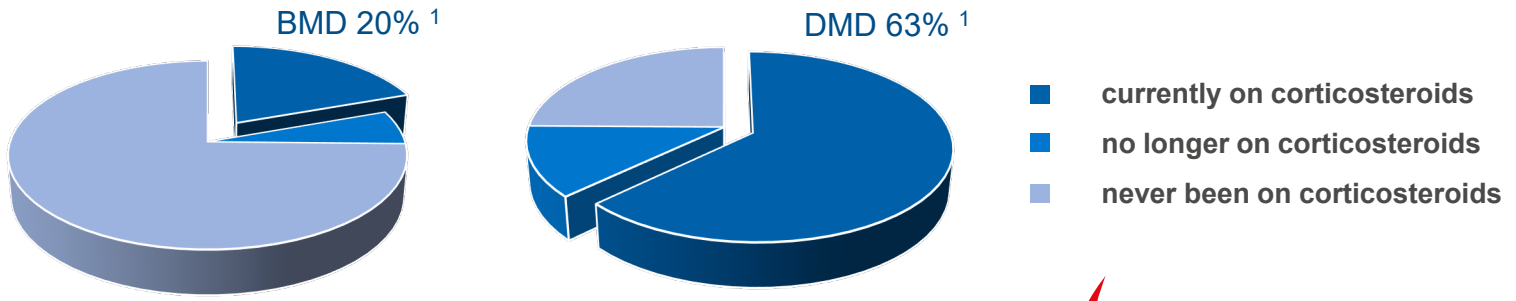
**CORTICOSTEROIDS IN BMD**

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

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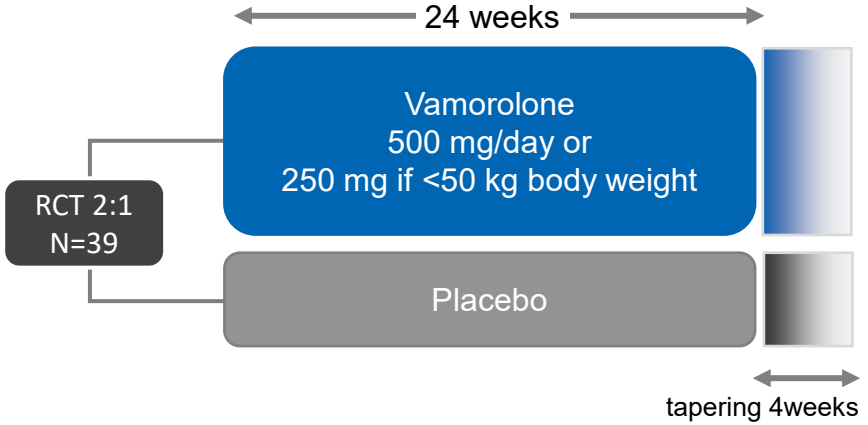
# Vamorolone holds promise in BMD based on data generated for DMD

## 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

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# Half-year 2024 financial results and update (Sept 12, 2024)


**Santhera Announces Half-Year 2024 Financial Results and Provides Corporate Update**

- Revenue from contracts with customers of CHF 14.1 million (H1-2023: CHF 3.9 million)
- Operating result of CHF -17.7 million (H1-2023: CHF -20.3 million) and net result of CHF -15.3 million (H1-2023: CHF -23.3 million)
- AGAMREE® (vamorolone) launched in Germany and Austria as first European markets; North America partner has launched in the U.S.
- Approval of AGAMREE in the UK for the treatment of Duchenne muscular dystrophy (DMD); new drug application (NDA) in DMD under regulatory priority review in China
- Cash and cash equivalents of CHF 16.5 million (June 30, 2024); bolstered by financing of up to CHF 69 million (closed in August 2024) to provide funding into 2026 when cash flow break-even is expected
- Business now fully focused on European commercialization and further geographic expansion of AGAMREE in DMD

**Pratteln, Switzerland, September 12, 2024 – Santhera Pharmaceuticals (SIX: SANN) announces the Company's financial results for the six months ended June 30, 2024, reports on progress with AGAMREE® (vamorolone) for the treatment of Duchenne muscular dystrophy (DMD) and provides updates on its corporate and financing initiatives.**


https://www.santhera.com/assets/files/financial_reports/2023_SANN_AnnualReport2023_final.pdf

# Santhera financial status

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

- **Cash runway**
  - August 2024: CHF 52 million post financing and settlement of convertible bonds
  - 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**
  - 07-2023: North American licensing to Catalyst
  - 01-2024: Launch in Germany on Jan 15, 2024
  - 03-2024: Launch in U.S. on Mar 13, 2024
  - 03-2024: NDA filed in China by partner Sperogenix
  - 08-2024: Up to CHF 69 million financing extending runway
  - 09-2024: Distribution partner Genesis for Eastern EU
  - 09-2024: MAA accepted for review by Swissmedic

- **Capital structure**
  - Basic shares outstanding 13.0 million
  - Market capitalization CHF 123 million (per share CHF 9.49)
  - Major shareholders Catalyst (11%), Idorsia (10%) and Highbridge (8%)
  - Research by H.C. Wainwright, Octavian and ValuationLAB

*All amounts consolidated IFRS and unaudited



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# Santhera Pharmaceuticals

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

October 1, 2024