Santhera Pharmaceuticals

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

Corporate Presentation

January 17, 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 EU core countries

Strong rare disease development capabilities

Three approvals for AGAMREE® (vamorolone) in Duchenne muscular dystrophy

Approved by FDA (10/2023), EMA/EC (12/2023) and MHRA (01/2024) for use in DMD

Differentiated safety profile addresses needs across broad DMD patient segments

Potential as alternative to corticosteroids in range of other therapeutic indications

Neutrophil elastase inhibitor Ionodelestat Phase 2 ready in pulmonary indications

Novel anti-inflammatory agent for neutrophil associated pulmonary disorders in general

Finance

U.S. license deal with Catalyst Pharmaceuticals in 2023 valued at up to USD 231 million plus royalties

Cash runway into 2025; Major shareholders: Catalyst Pharmaceuticals, Inc. 11.2%; Idorsia 10.3%

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Santhera pipeline with two assets and broad therapeutic potential

Opportunities beyond current active program in Duchenne muscular dystrophy (DMD)

AGAMREE® (vamorolone) foundational therapy in DMD

- U.S. FDA full approval on October 26, 2023
- 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 in top-5 Europe (Germany, UK, France, Italy, Spain), plus Benelux, Austria, Switzerland. Commercialization in other countries via partner(s)
- Peak potential > EUR 150 million in DMD (in Santhera own markets)[1]
- Commercialization in the U.S. 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; MAA: marketing authorization application;
MAD: Multiple Ascending Dose; CF: Cystic Fibrosis; ARDS: Acute Respiratory Distress Syndrome

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

Launch in Germany as of Jan 15, 2024, and expected launch in Q1-2024 in the U.S.

Molecule	Indication	IND Ph 1 PoC Pivotal Filing Market	Milestones and remarks
Vamorolone[1] • dissociative steroid • oral suspension	**Duchenne muscular dystrophy**	Approved in US, EU and UK	North America & China partnerships Catalyst pharmaceuticals 曙方医药 Sperogenix THERAPEUTICS
	Becker muscular dystrophy		Trial under FDA grant to partner ReveraGen BioPharma
	Steroid alternative in multiple pediatric rare indications		Under evaluation
Lonodelestat[2] • 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

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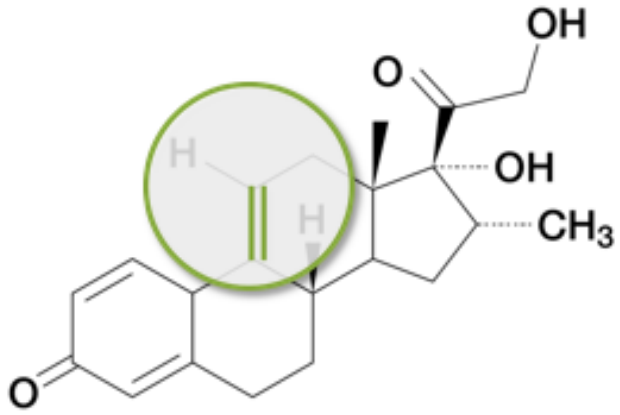
AGAMREE® (vamorolone) in Duchenne muscular dystrophy and potentially other inflammatory disorders



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
- Newer therapies likely to be used in combination with corticosteroids

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

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

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 U.S. (Emflaza®), achieves attractive margins

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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.

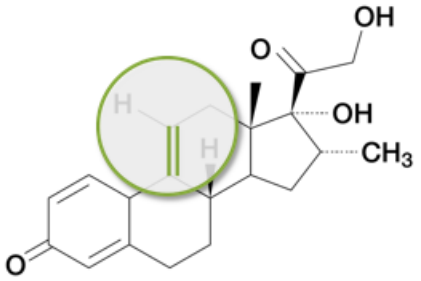
TOO LATE
TOO LITTLE
TOO SOON

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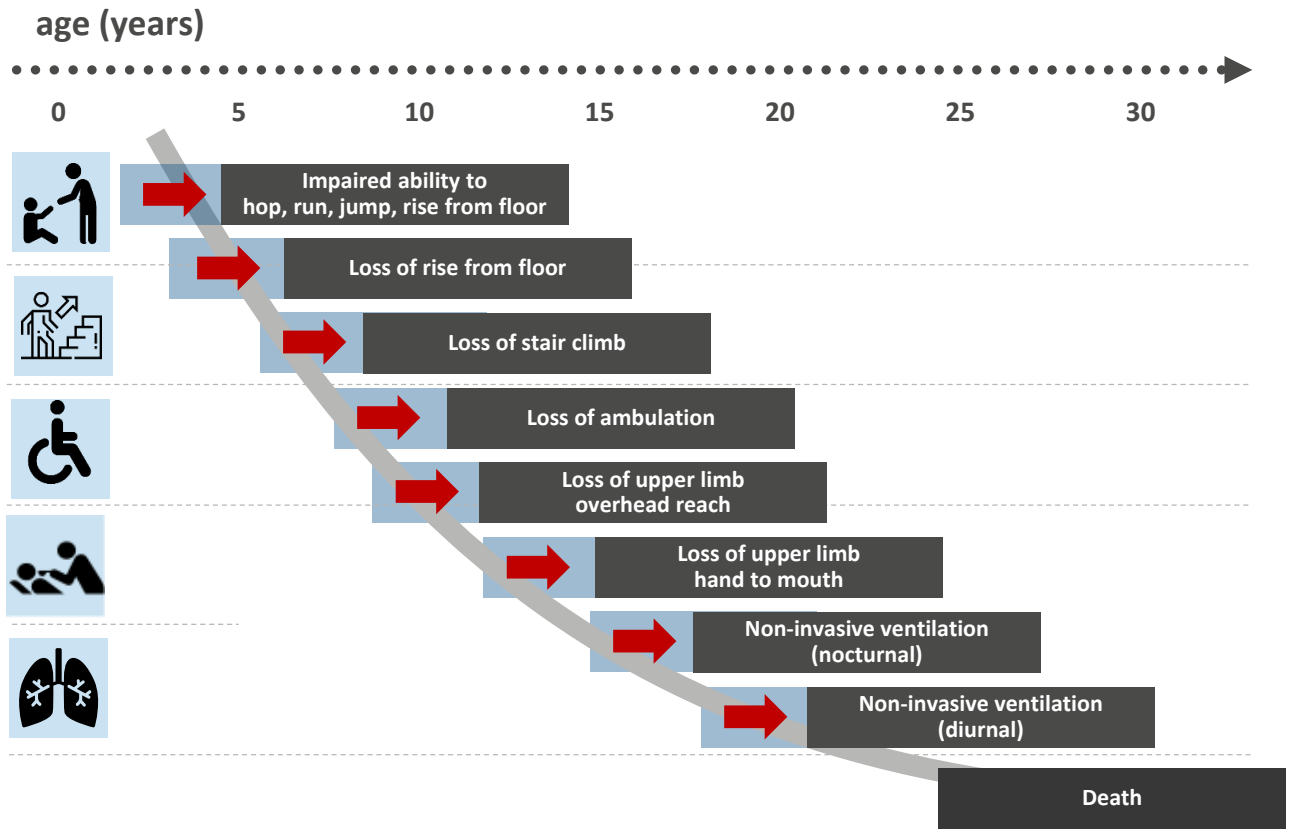
Corticosteroids delay disease progression in DMD by 2 – 3 years[4,6]

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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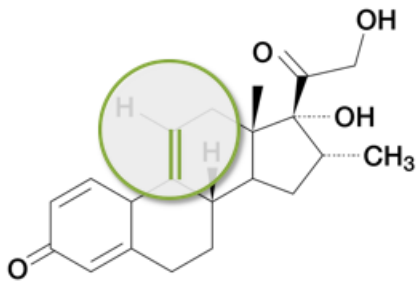
AGAMREE® (vamorolone) dissociative properties

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

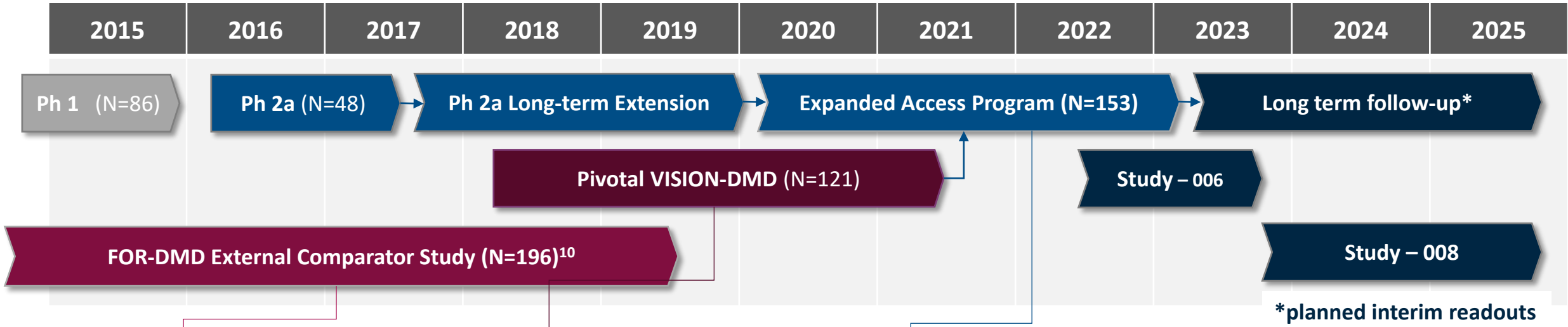
Unlike corticosteroids, potential for reduction of steroid-associated side effects

- 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)

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

Study-008:
effectiveness in 7–18 year-old ambulatory / non-ambulatory

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; ** Studies as part of pediatric investigational plan (PIP)

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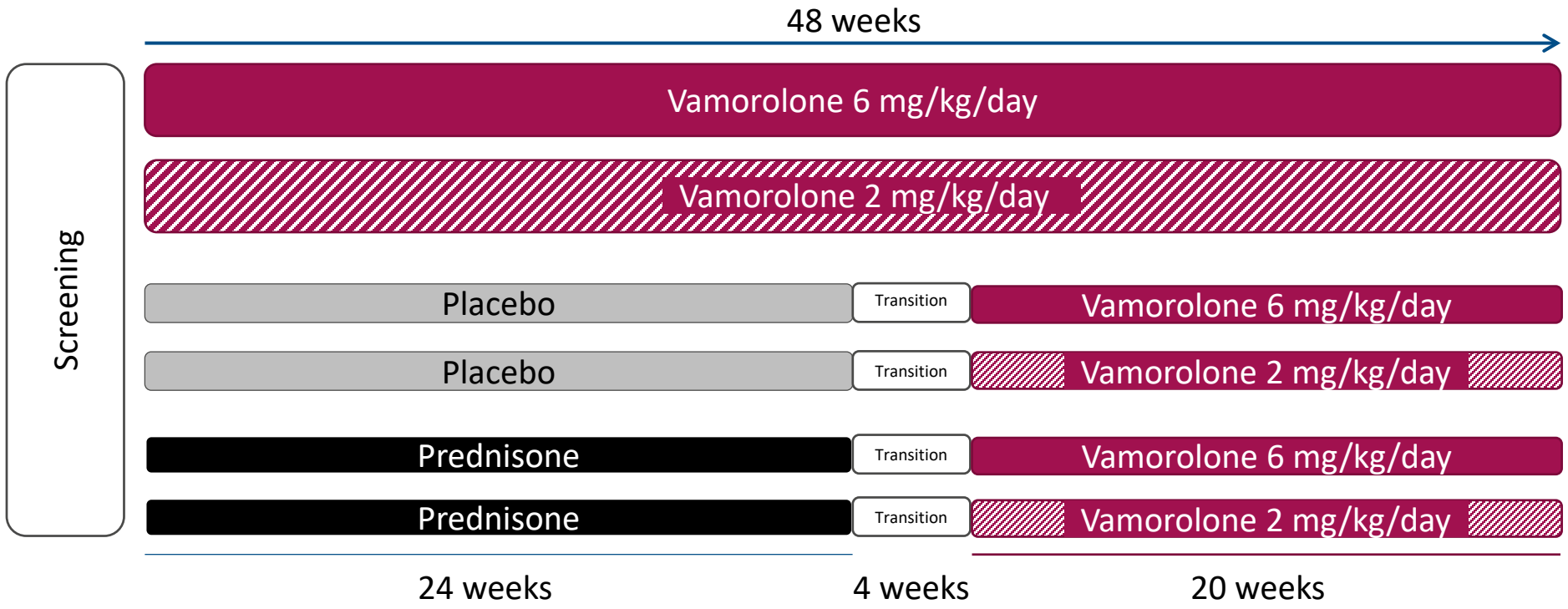
Pivotal VISION-DMD: Study design

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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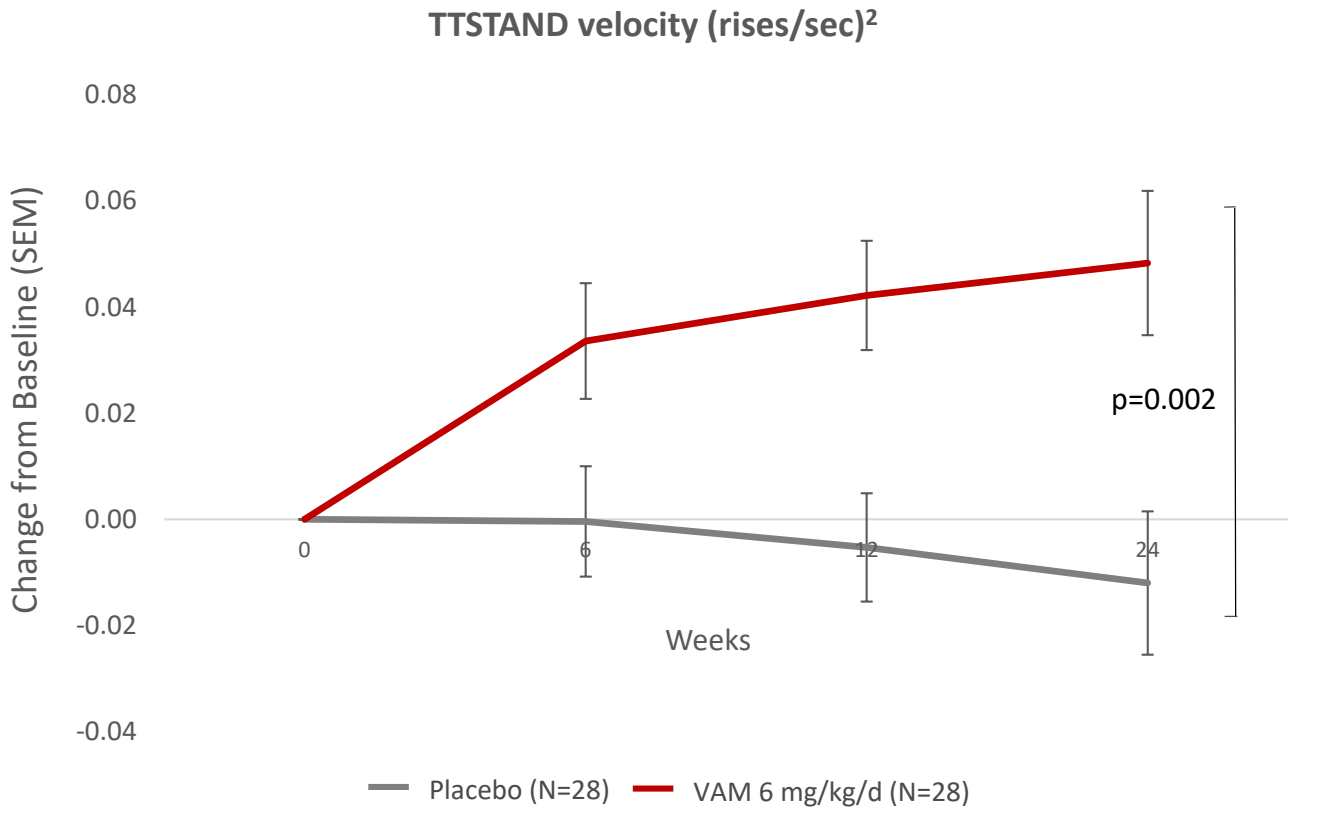
Primary endpoint met with clinically relevant treatment difference

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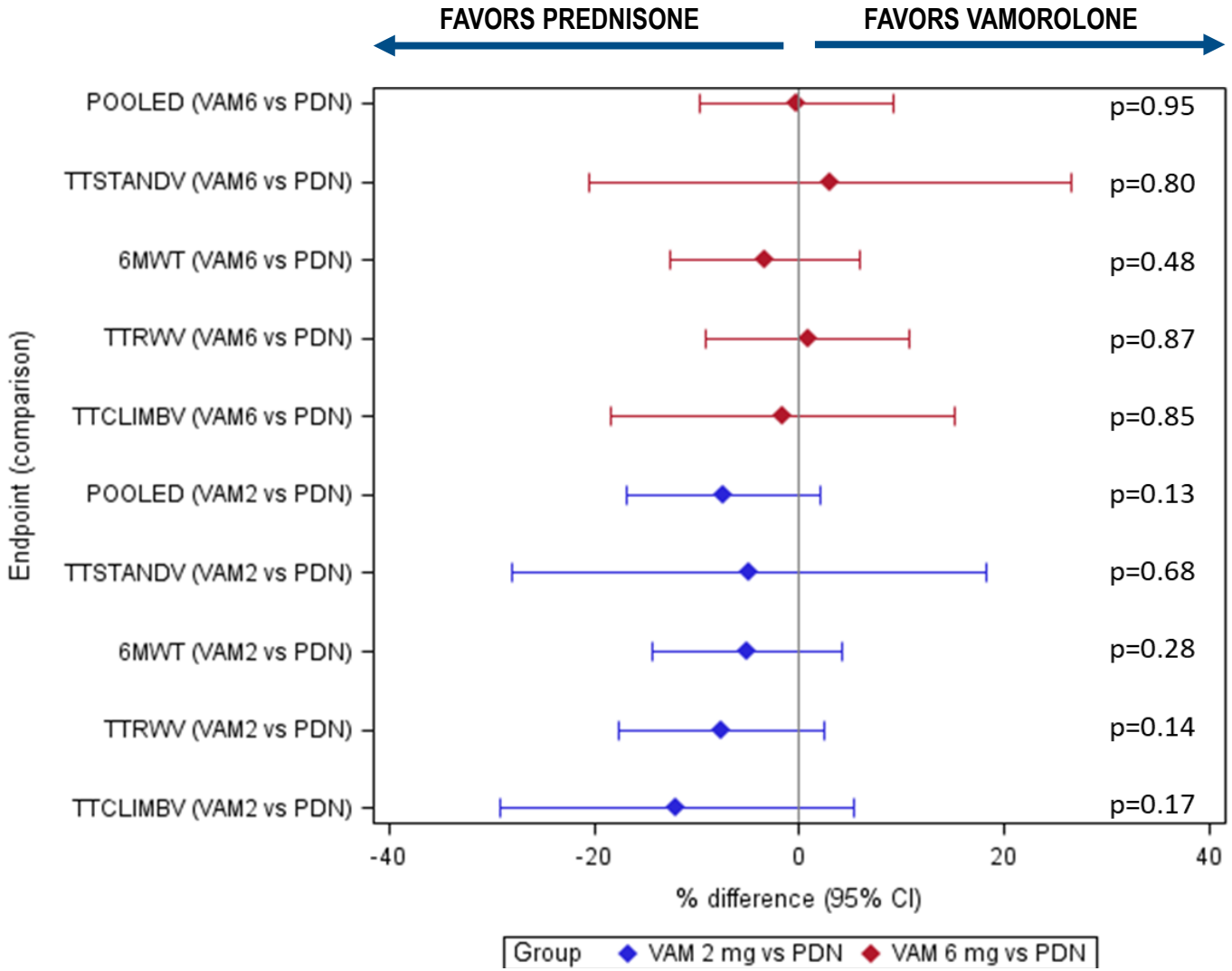
Comparable efficacy of vamorolone 6 mg/kg/d vs prednisone 0.75 mg/kg/d

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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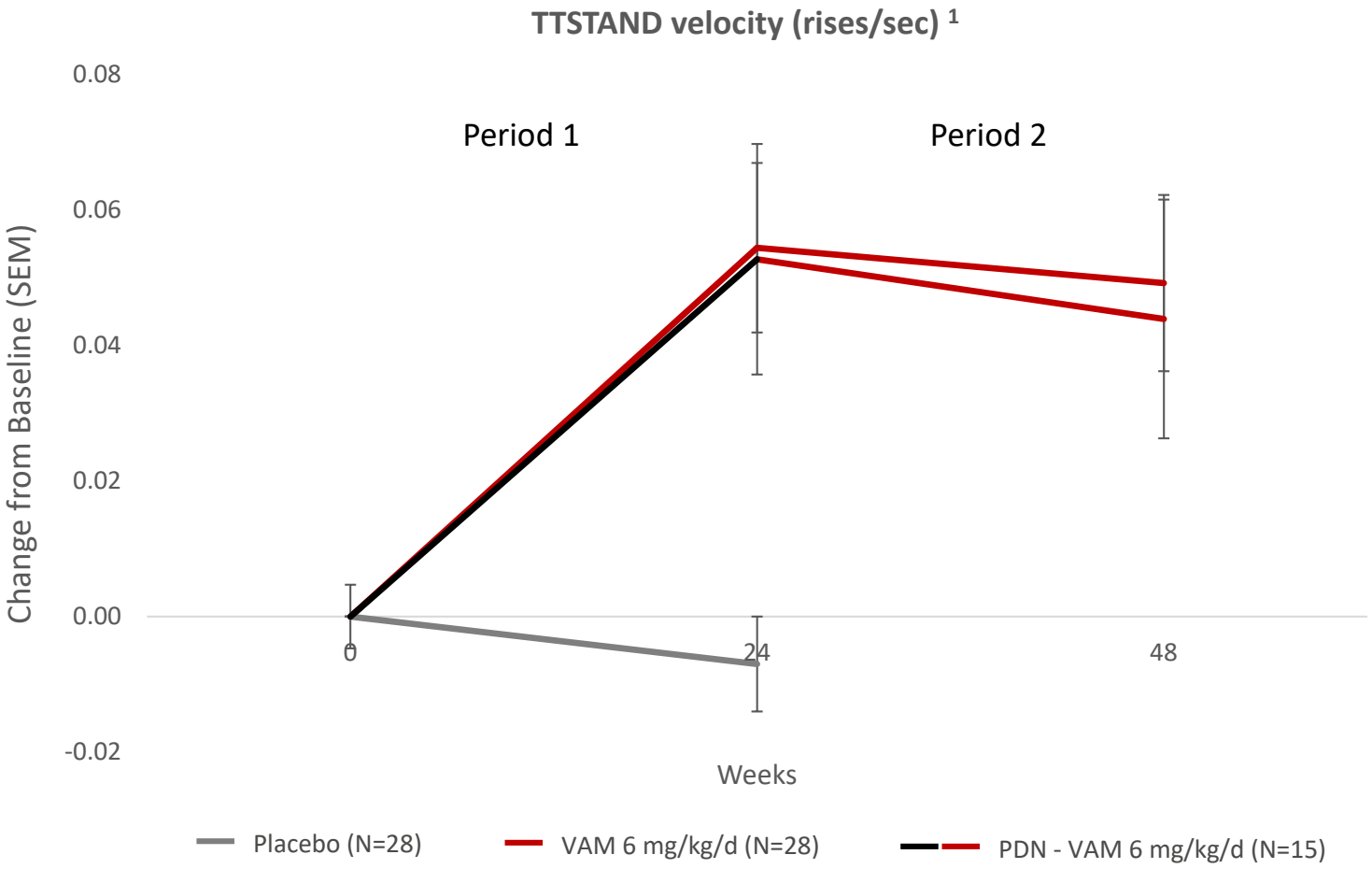
No loss of efficacy when switching from prednisone to vamorolone

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]

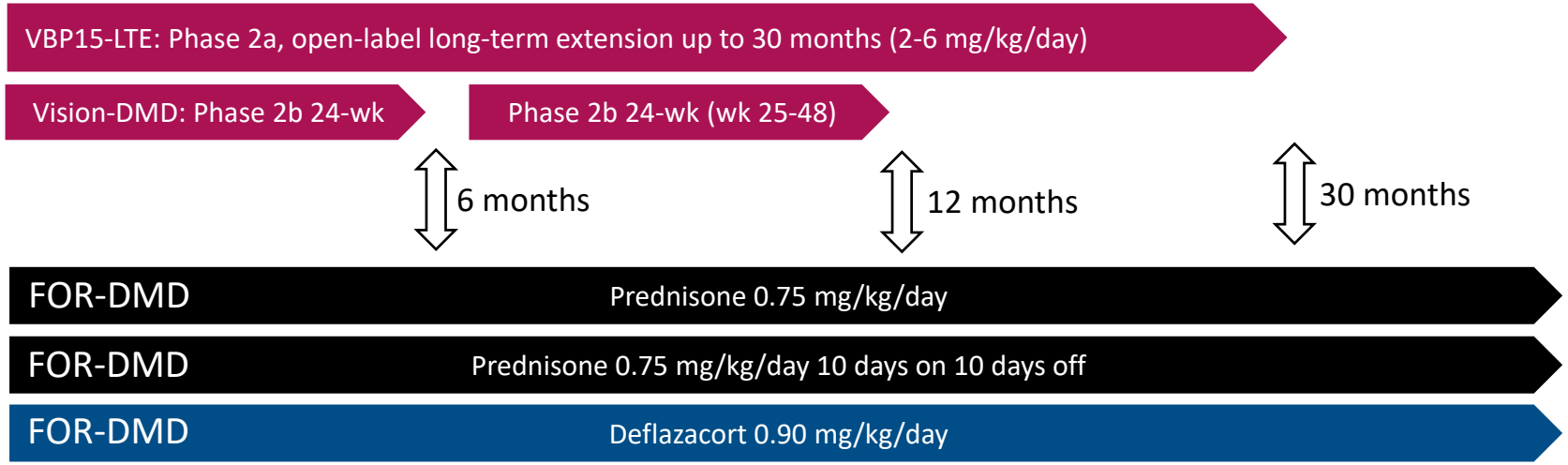
1. Data on File VAM-2021-002, mITT-2: modified intention to treat population from period 1 and 2, MMRM estimates of changes from baseline. PDN: prednisone 0.75mg/kg/day, PDN-VAM: prednisone 0.75 mg/kg/d in Period 1 transitioned to vamorolone 6mg/kg/d in Period 2 group after a 4-week tapering period;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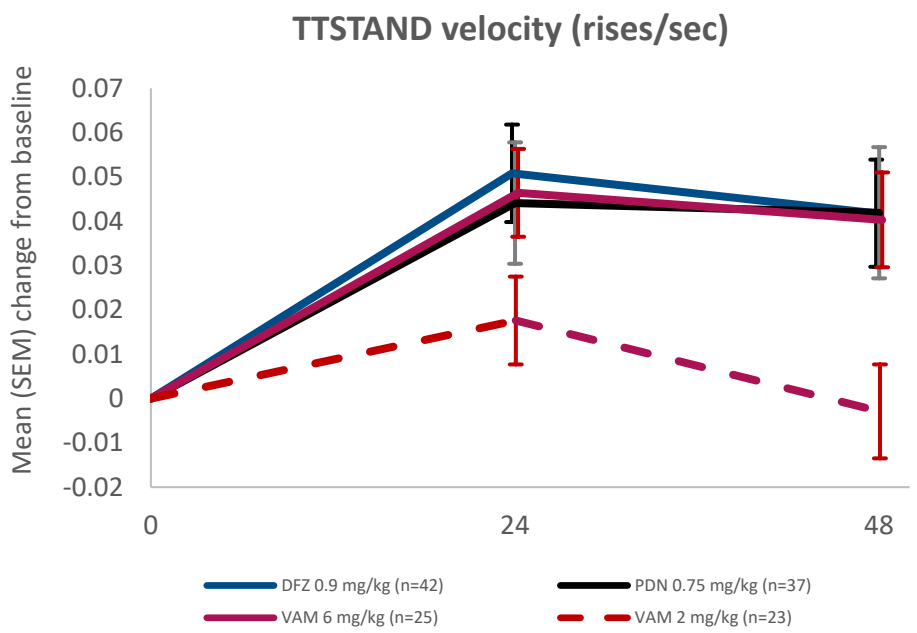
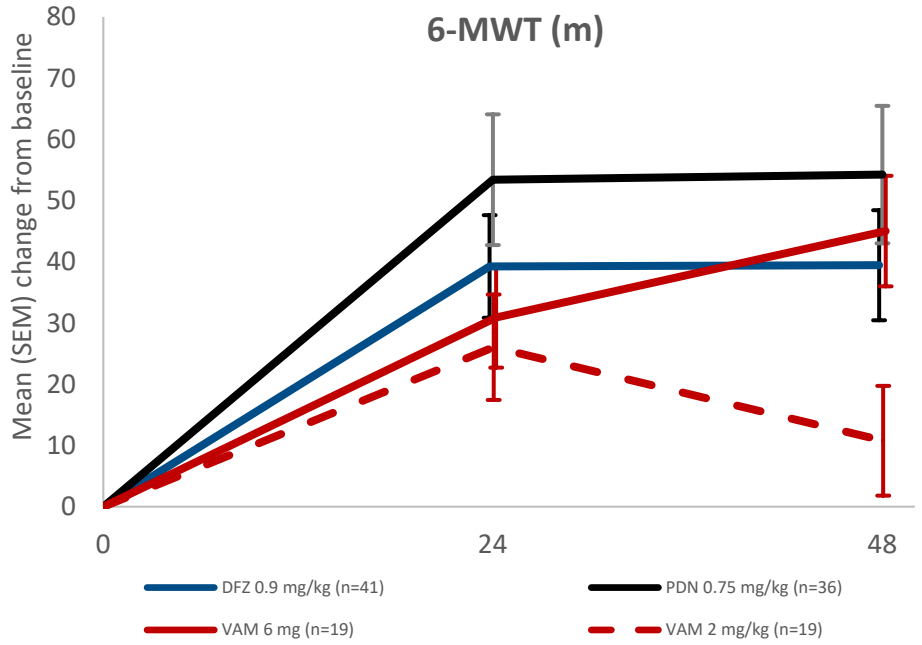
VISION-DMD pre-specified* analyses vs FOR-DMD external control

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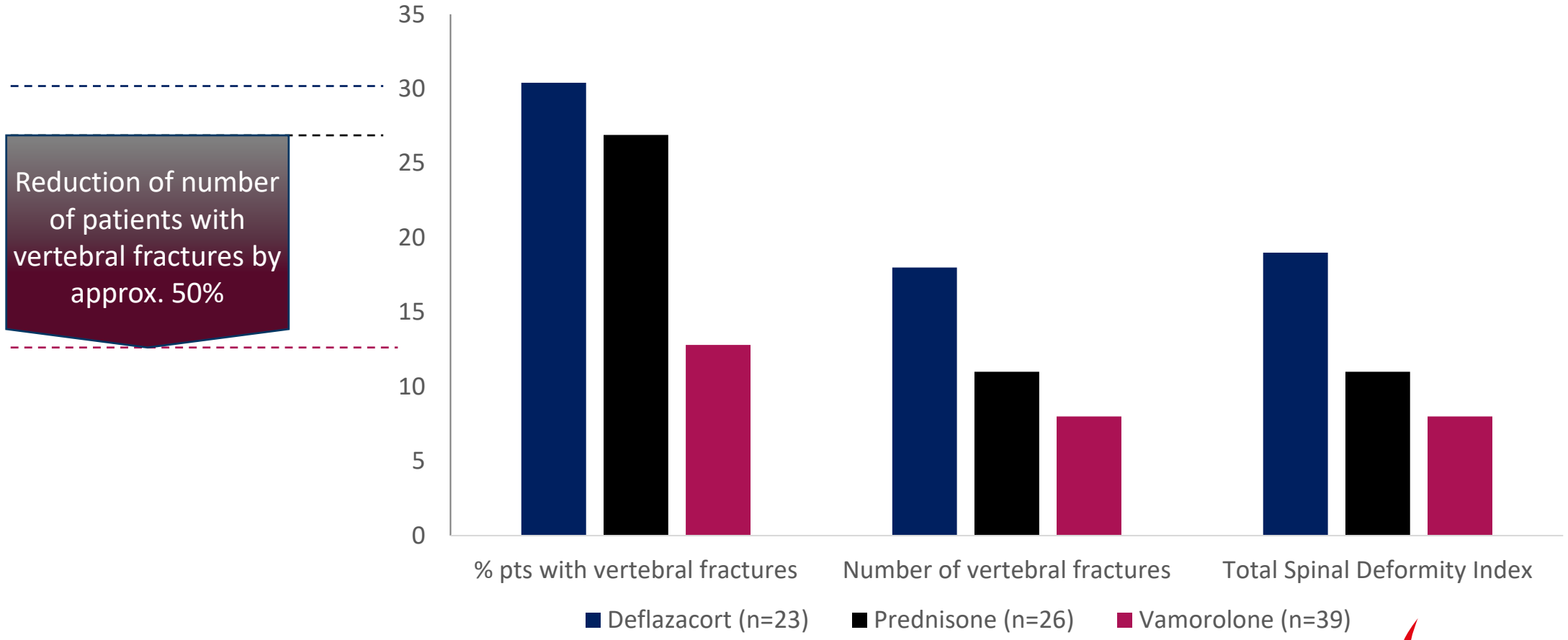
Fewer and less severe spinal fractures with vamorolone compared to classical corticosteroids over 2.5 years

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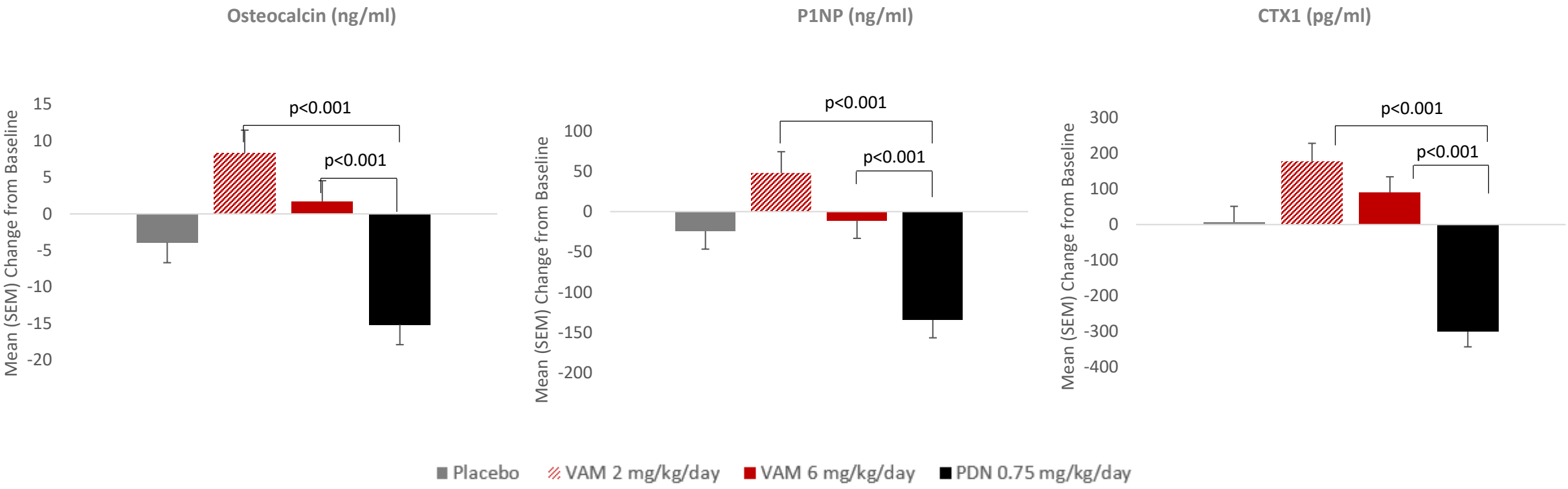
Bone biomarker data from VISION-DMD study supports findings on long-term bone health

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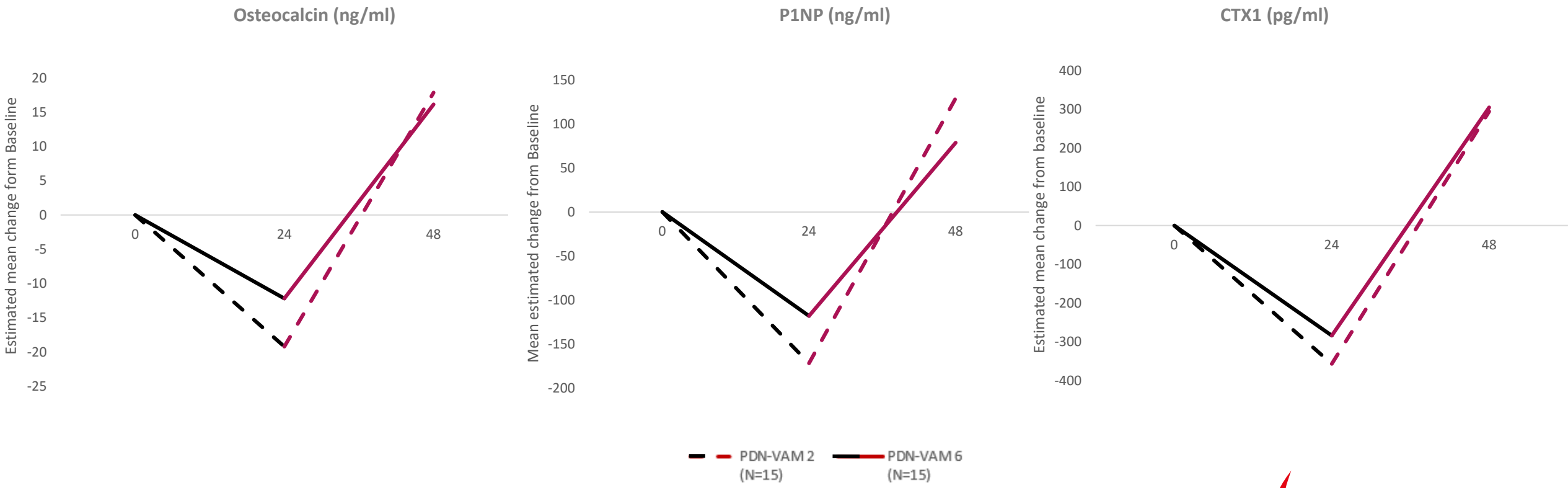
Bone biomarker data from VISION-DMD study supports findings on long-term bone health

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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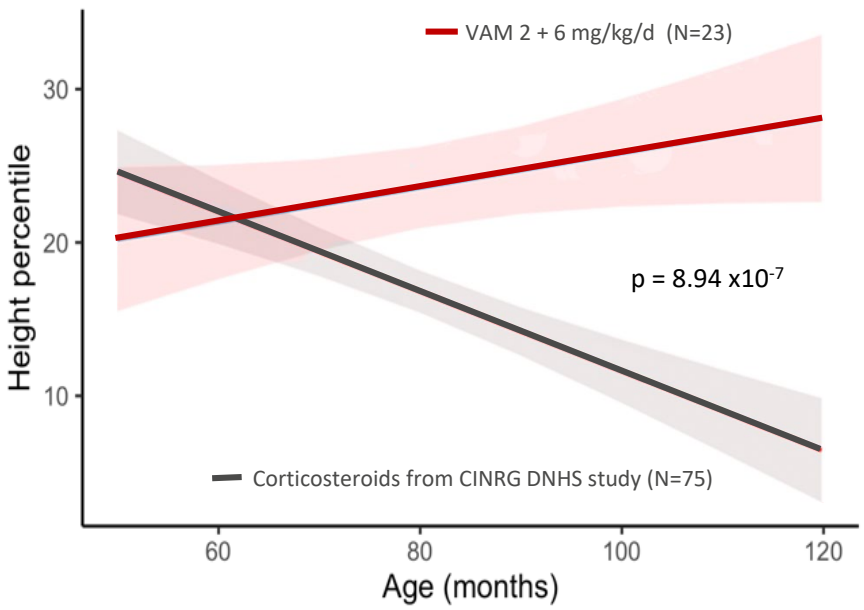
Vamorolone allows for normal bone development and growth

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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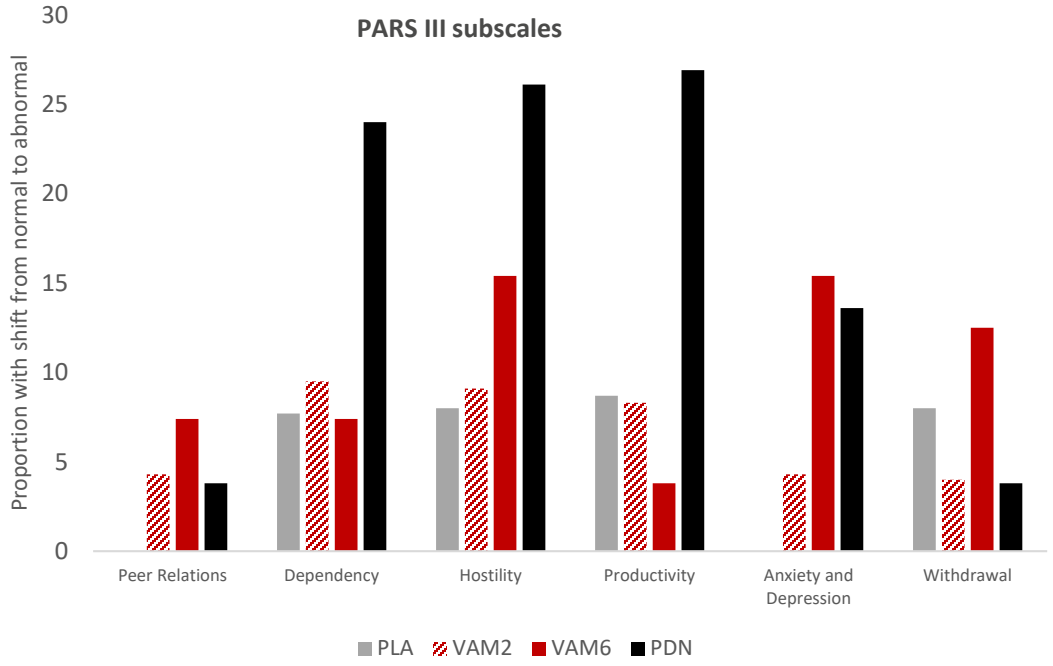
Fewer and less severe behavioral problems reported with vamorolone

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® 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 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 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**

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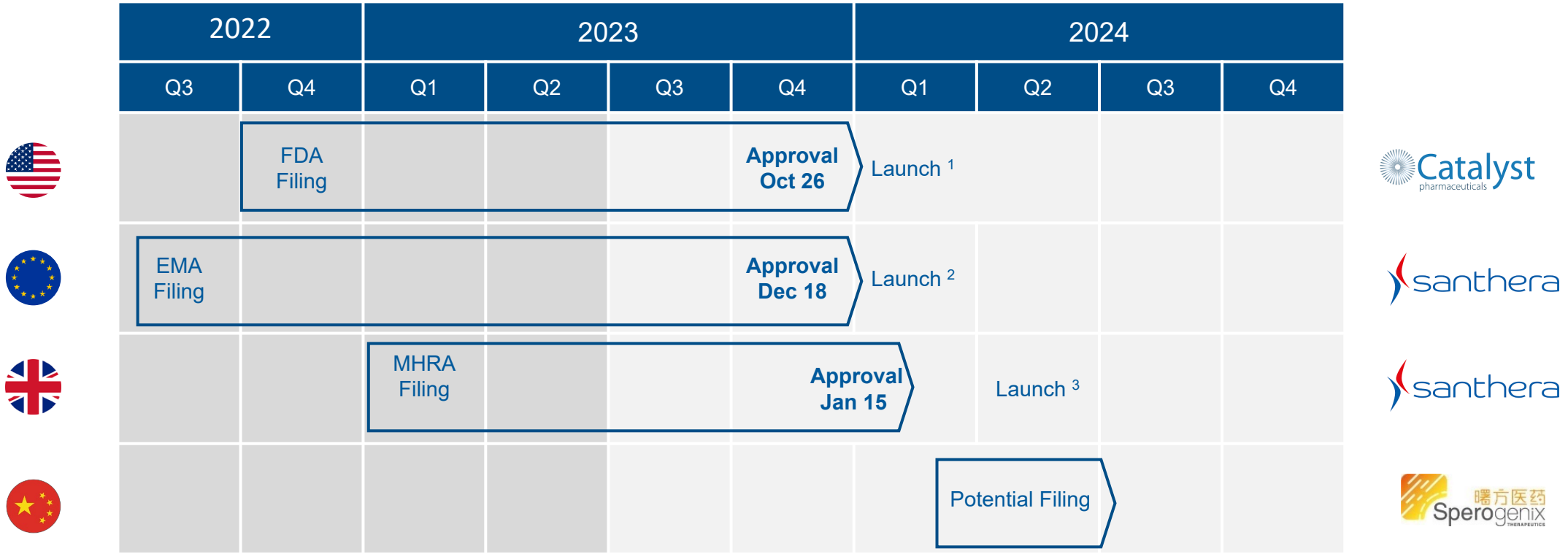
Full approval by FDA, EMA and MHRA for AGAMREE® in DMD

- Approvals for all patients of age 2 (US) or 4 (EU, UK) years and older; launch in Germany as of Jan 15, 2024
- 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)



1: Expected through partner Catalyst; 2: Staggered launch with first country Germany; 3: Launch review by NICE (National Institute for Health and Care Excellence) evaluation; EMA: European Medicines Agency; MHRA: Medicines and Healthcare Products Regulatory Agency; FDA: Food and Drug Administration

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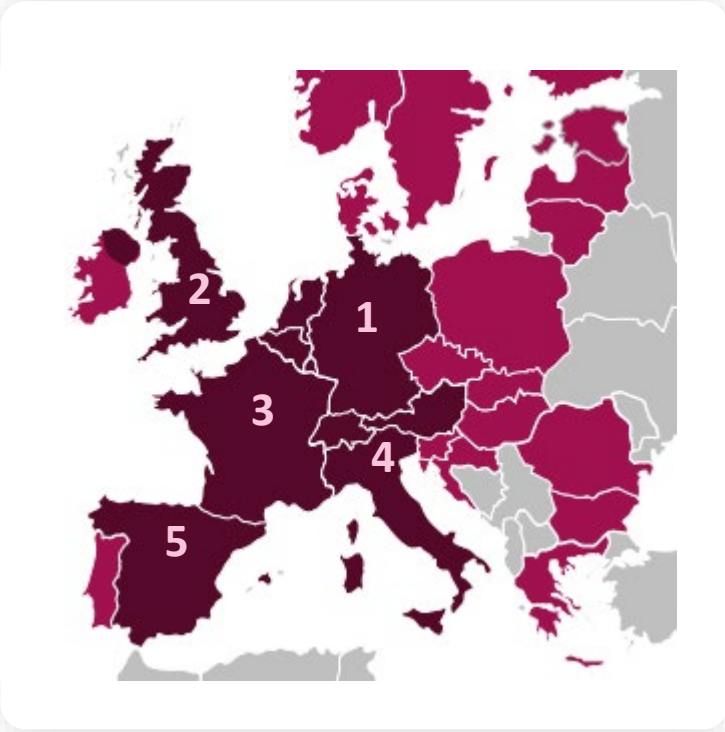
Santhera commercial launch in key European geographies

Santhera aims to market vamorolone in DMD itself in core territory with population of ~340 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 60 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 partners



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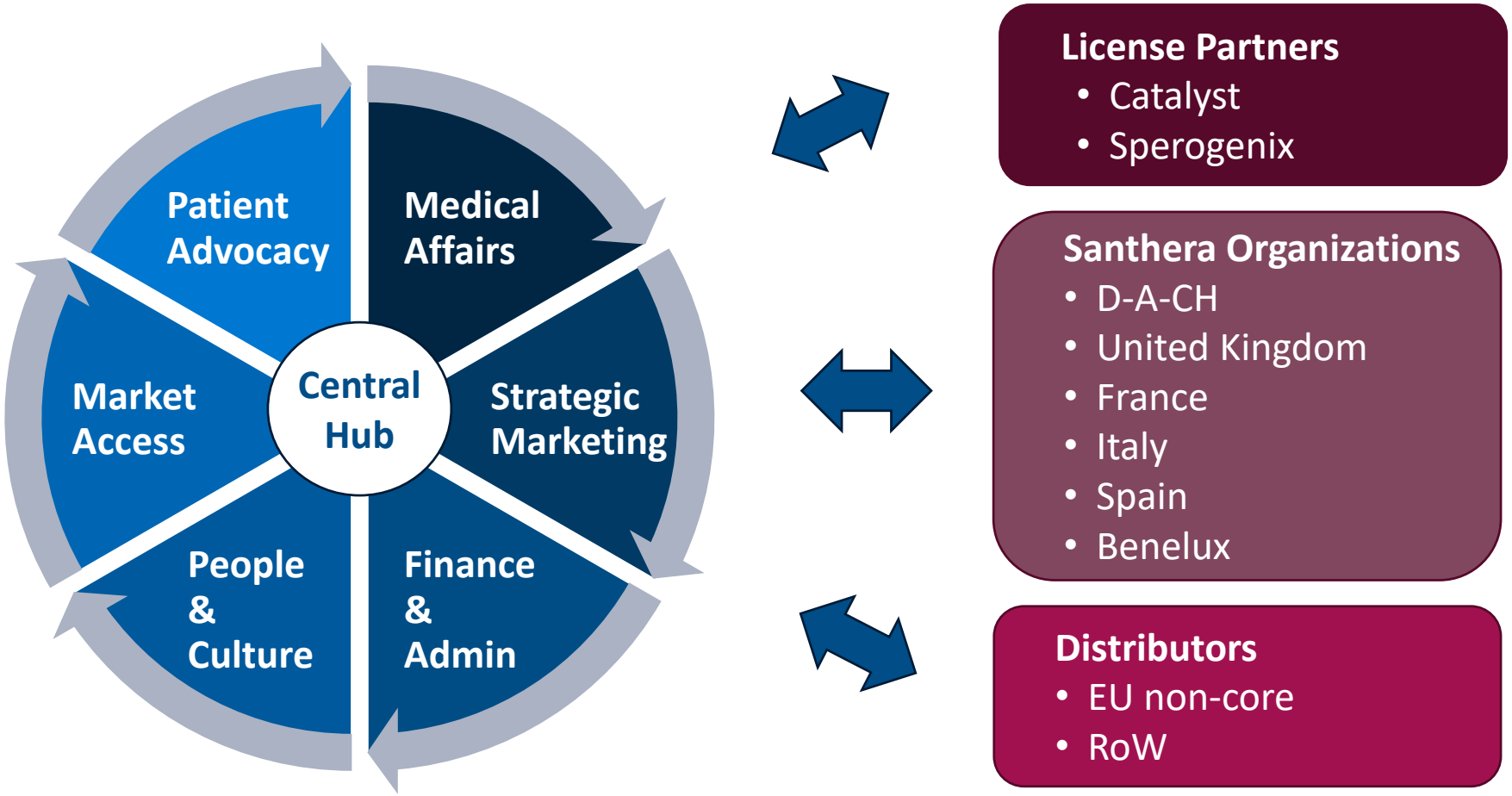
Santhera commercial set-up with central hub structure at headquarters

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



D-A-CH: Germany-Austria-Switzerland; Benelux: Belgium, Netherlands, Luxembourg

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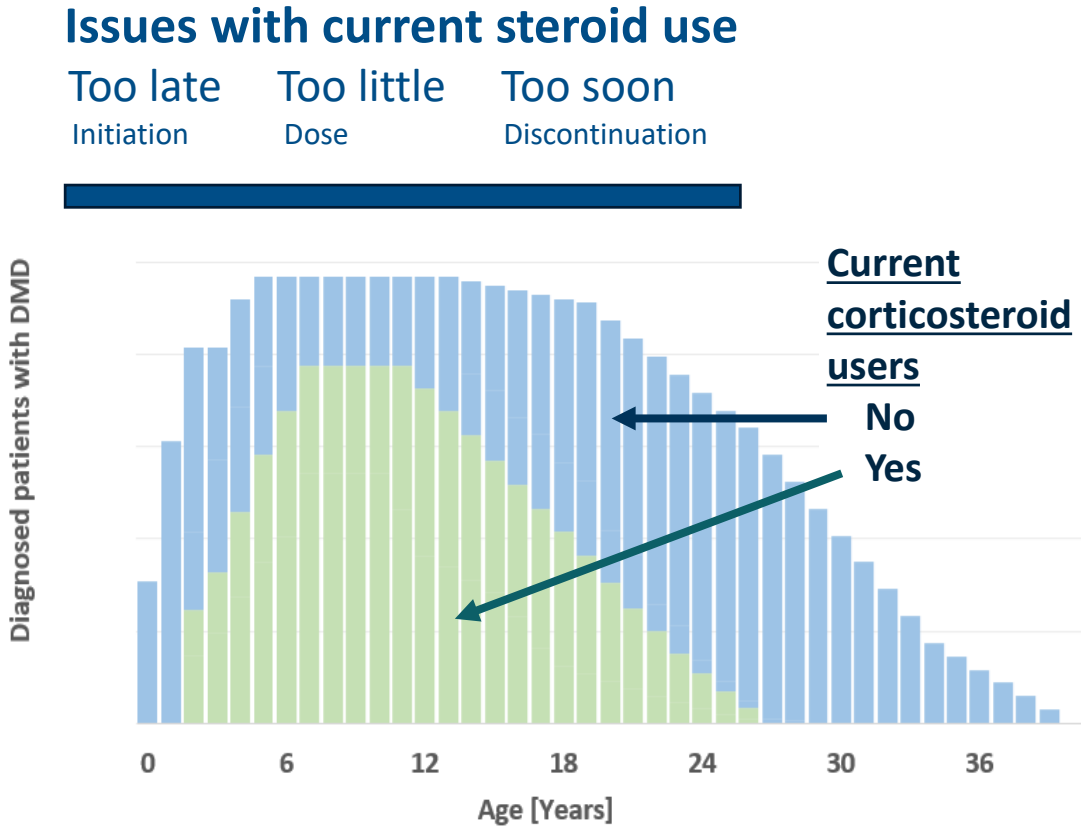
Market opportunity to change the foundational therapy in DMD

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

- **Current corticosteroid use**
  - With 60-70% of patients on steroid treatment, currently up to 8,000 boys/men are being treated with standard corticosteroids in the Santhera own commercialization markets* alone[1,2]
- **AGAMREE® opportunity for change**
  - Replacing current corticosteroid treatment initiation
  - Switching patients from standard corticosteroids
  - Restarting treatment for patients recently discontinued
- **Peak market size potential (Santhera)**
  - Estimated range of 3,000 to 4,000 patients on AGAMREE®
  - Standard range of orphan drug pricing leads to peak sales estimate exceeding EUR >150 million



1: Cowen et al., 2019, BMC Neurology; 2: Ryder et al., 2017, Orphanet Journal Rare Diseases
* Germany-Austria-Switzerland, United Kingdom, France, Italy, Spain & Benelux (Population ~340 million)

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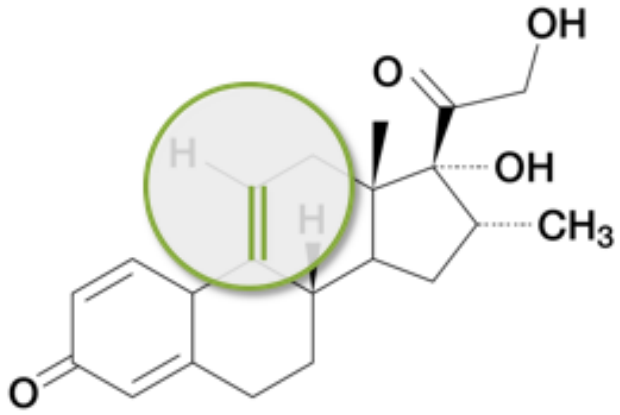
Vamorolone in Becker muscular dystrophy



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

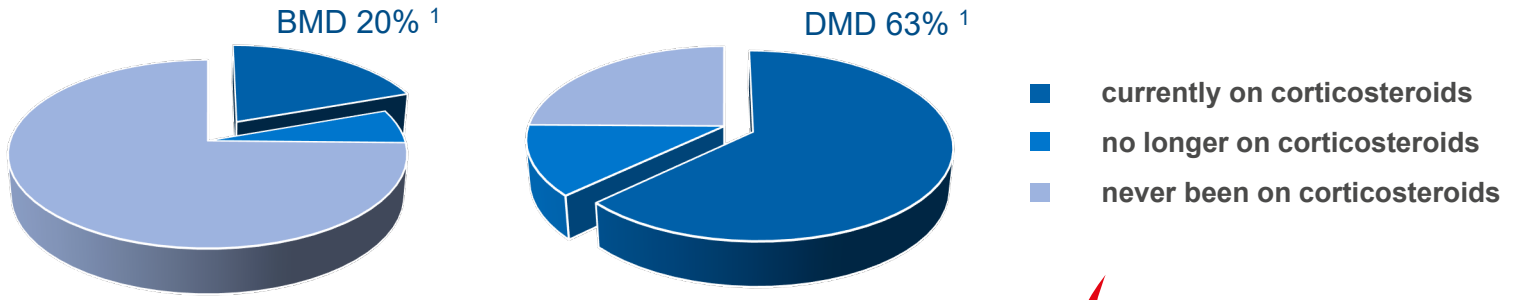
CORTICOSTEROIDS IN BMD

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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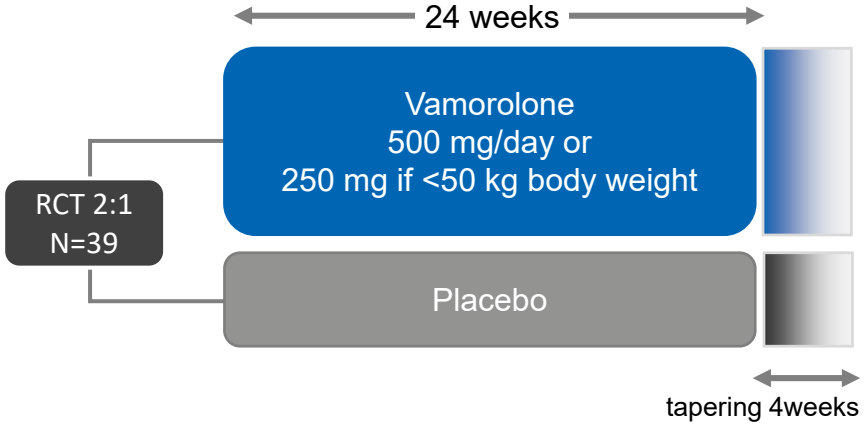
Vamorolone holds promise in BMD based on data generated for DMD

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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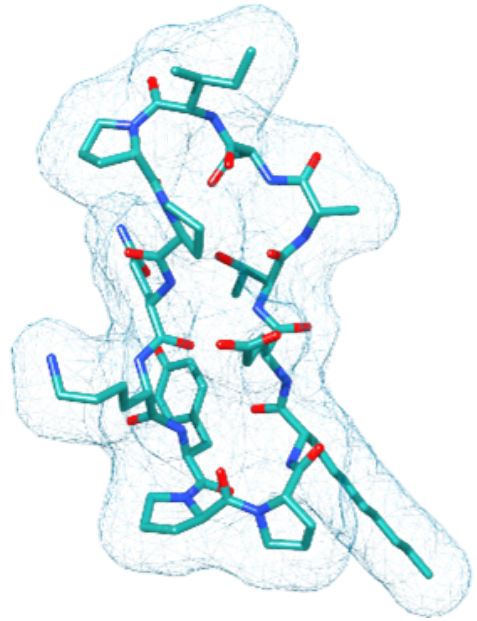
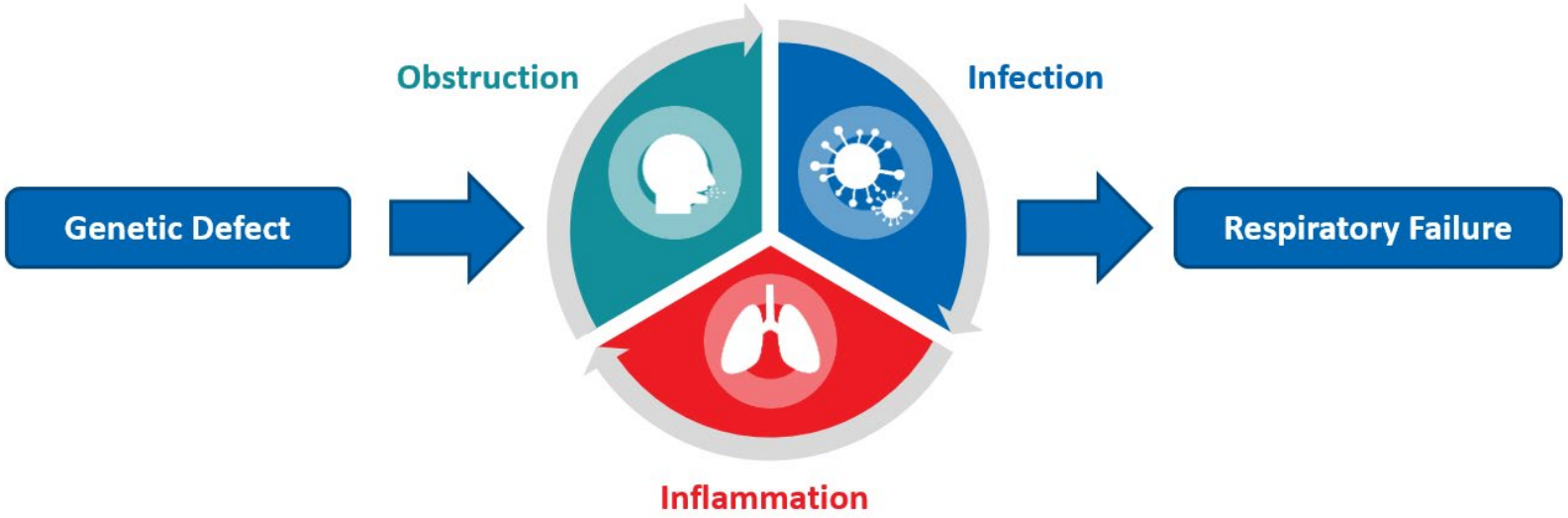
Lonodelestat in cystic fibrosis and potentially other inflammatory pulmonary disorders



Cystic fibrosis is a rare genetic lung disorder with unmet medical need

Genetics	Cause	Patients	Symptoms	Medical need
Autosomal recessive disorder diagnosed at young age	Mutations in the CF transmembrane conductance regulator (CFTR) gene	More than 80,000 patients in U.S. and Europe combined	Persistent lung infections, chronic inflammation and loss of respiratory function	No approved treatment specifically addressing inflammation in CF

Need to break vicious cycle of airway obstruction, respiratory failure and resulting chronic inflammation[1,2]



1. De Rose, V, *Eur Resp J* (2002) 19;333; 2. Mogayzel, PJ, et. al. *Am J Respir Crit Care Med (2013)* 187(7):680

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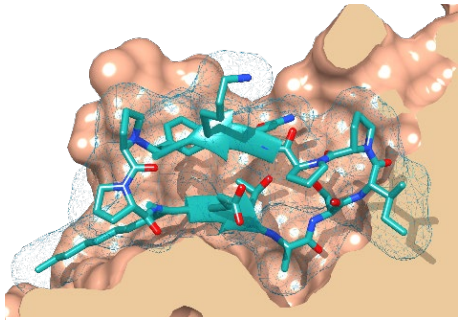
Lonodelestat targets elastase, a protease responsible for lung damage

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)



Lonodelestat bound to elastase

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

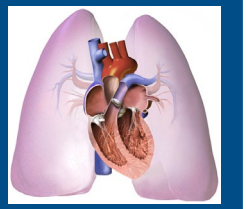
Opportunities beyond CF

- 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

Santhera financial status

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

- **Key figures (CHF million* as of June 30, prior closing Catalyst agreement in July 2023)**
  - Net (loss) for the period (23.3)
  - Cash (used) in operations (15.5)
  - Cash & cash equivalents 1.7
  - Debt outstanding (maturity 2024) ** (49.1)
  - Shareholders' equity (42.8)

- **Capital structure**
  - Basic shares outstanding 12.6 million
  - Market capitalization CHF 126 million (per share CHF 10)
  - Major shareholders Catalyst (11.2%) and Idorsia (10.3%)
  - Research by H.C. Wainwright, Octavian and valuationLAB

- **Recent milestones AGAMREE®**
  - 07-2023: North American licensing to Catalyst
  - 10-2023: US approval by FDA
  - 12-2023: EU approval by European Commission
  - 01-2024: UK approval by MHRA
  - 01-2024: Launch in Germany on Jan 15, 2024

- **Upcoming milestones AGAMREE®**
  - Q1-2024: Commercial launch by Catalyst (U.S.)

- **Cash runway**
  - Into 2025 incl. commercial EU infrastructure & launch

*All amounts unaudited consolidated IFRS ; ** Includes Listed Convertible Bond Maturing Aug-24 CHF 13.5m

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Santhera Pharmaceuticals

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

January 17, 2024