Santhera Pharmaceuticals

Investor call – Looking ahead to 2022

December 8, 2021

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Vamorolone

48-week topline results from pivotal VISION-DMD study

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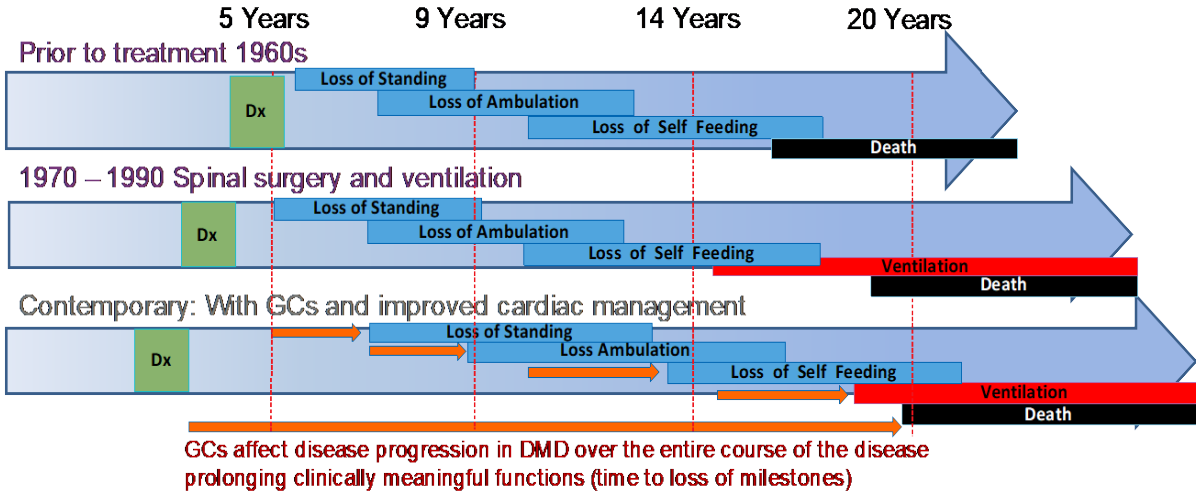
Corticosteroids are a standard of care for the treatment of DMD



If started early and continued, they delay time to loss of functional milestones by ~2 to 3 years across all stages of the condition

… up to 65% of patient discontinue treatment due to adverse events[3-5]

The most common are:

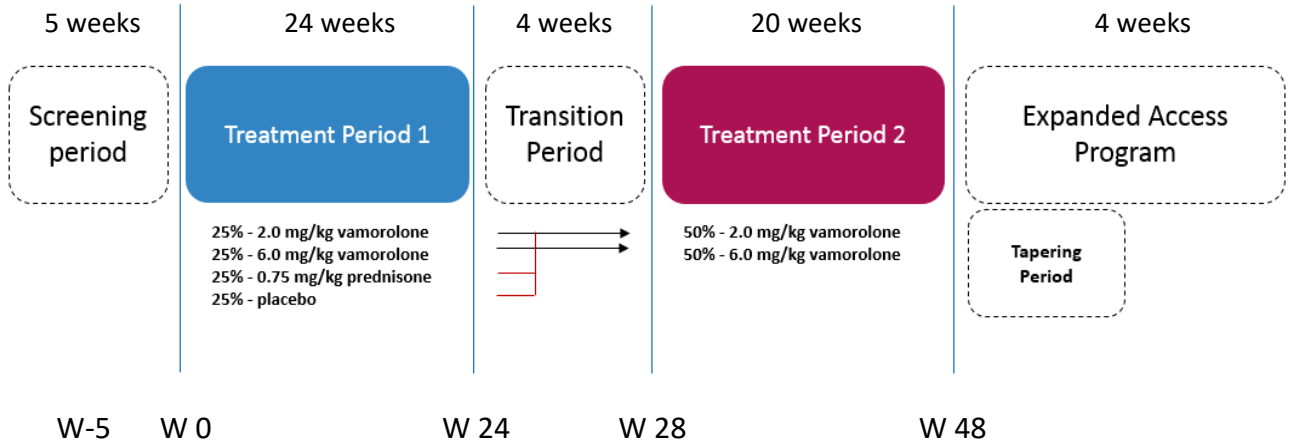
37%		Growth failure/delay[4,5]
37%		Behavioral changes[5]
30%		Bone changes or fractures[4,5]
55%		Cushingoid appearance[5]
65%		Weight gain[5]
31%		Excessive hair growth[4,5]
19%		Cataracts[4]

1. Bushby K and Connor E. Clin Investig (Lond). 2011 Sep;1(9):1217-1235; 2. McDonald C, et al. Muscle Nerve. 2013;48(1):32–54; 3. Cowen L, et al. BMC Neurol. 2019;19:84; 4. Wong B, et al. Treatment Pediatr. 2017;182:296–303; 5. Bello L, et al. Neurology. 2015;85:1048–1055; McDonald CM et al., Lancet 2018, 3;391 (10119):451-461

VISION-DMD: Study Design

Randomized, double-blind, placebo and active control trial in 121 steroid-naive patients, aged 4 - 7 yrs old

- FDA & EMA: Primary analysis vs placebo at 24 weeks
- EMA : maintenance of treatment effect including external comparison to prednisone, long term safety
- Safety and tolerability of switching from prednisone to vamorolone
- 114/121 subjects continued into Period 2 (mITT-2)



Outcome measures at week 24

Primary efficacy outcome measure: TTSTAND velocity

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). All doses daily

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Objective of the 48-week analysis

Pre-NDA meeting with FDA

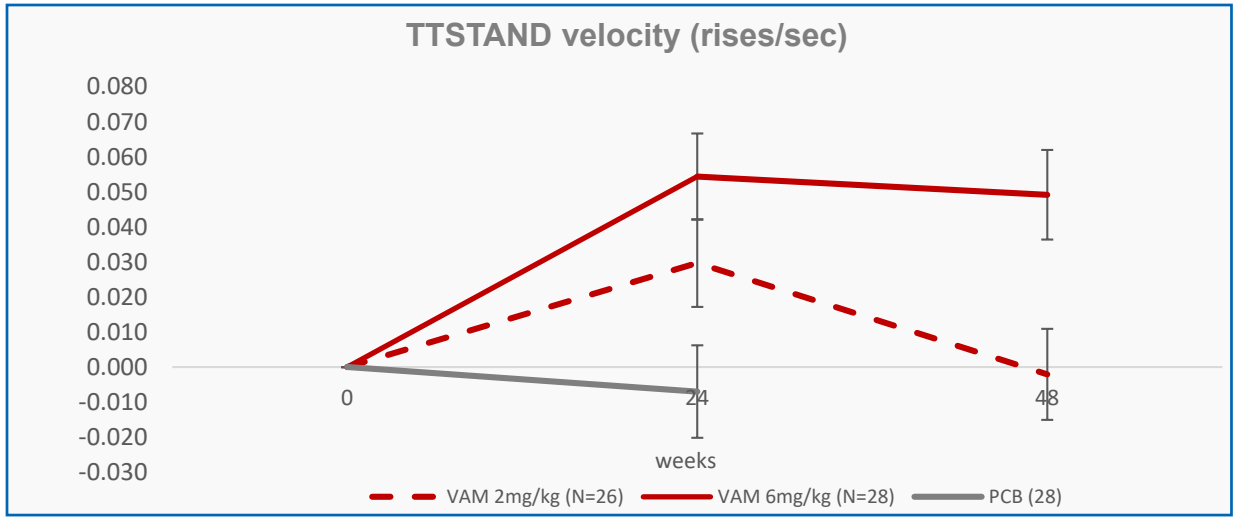
- Confirmed efficacy and safety **vs placebo** from 24-week data is sufficient to file
- Agreed on proposal to integrate 48-week and phase 2b open label data into the package

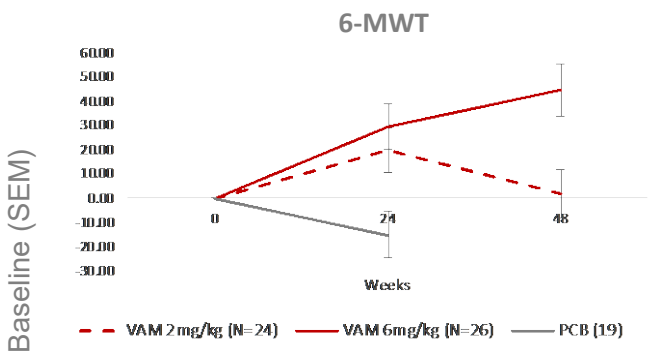
In addition, the 48-week analysis will provide further information on

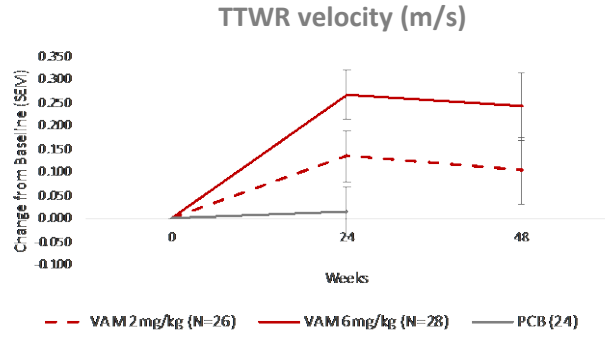
- On the proposed dosing regime
- Persistence of the treatment effect beyond 24 weeks
- Safety and effectiveness of switching from prednisone to vamorolone
- Longer term safety profile and differentiation of vamorolone

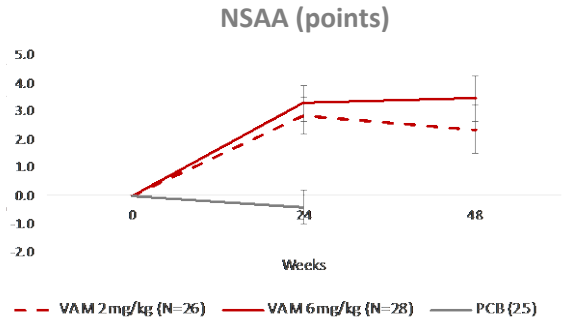
For EMA the submission will be driven by the 48-week dataset

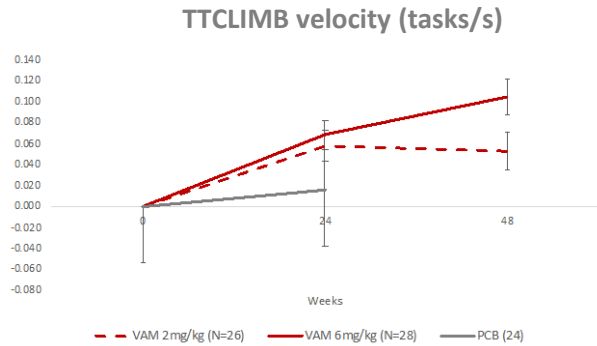
Improvements in timed function tests were maintained to week 48 with vamorolone 6mg/kg











All doses daily. MMRM estimates from mIIT-2 population

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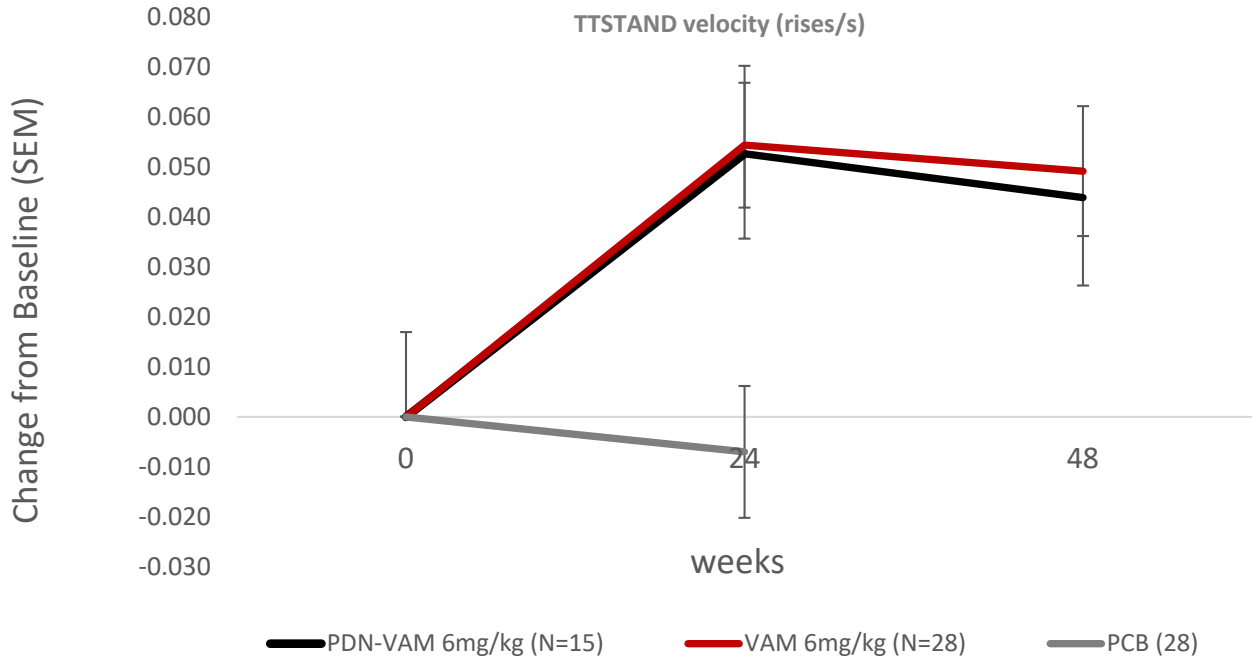
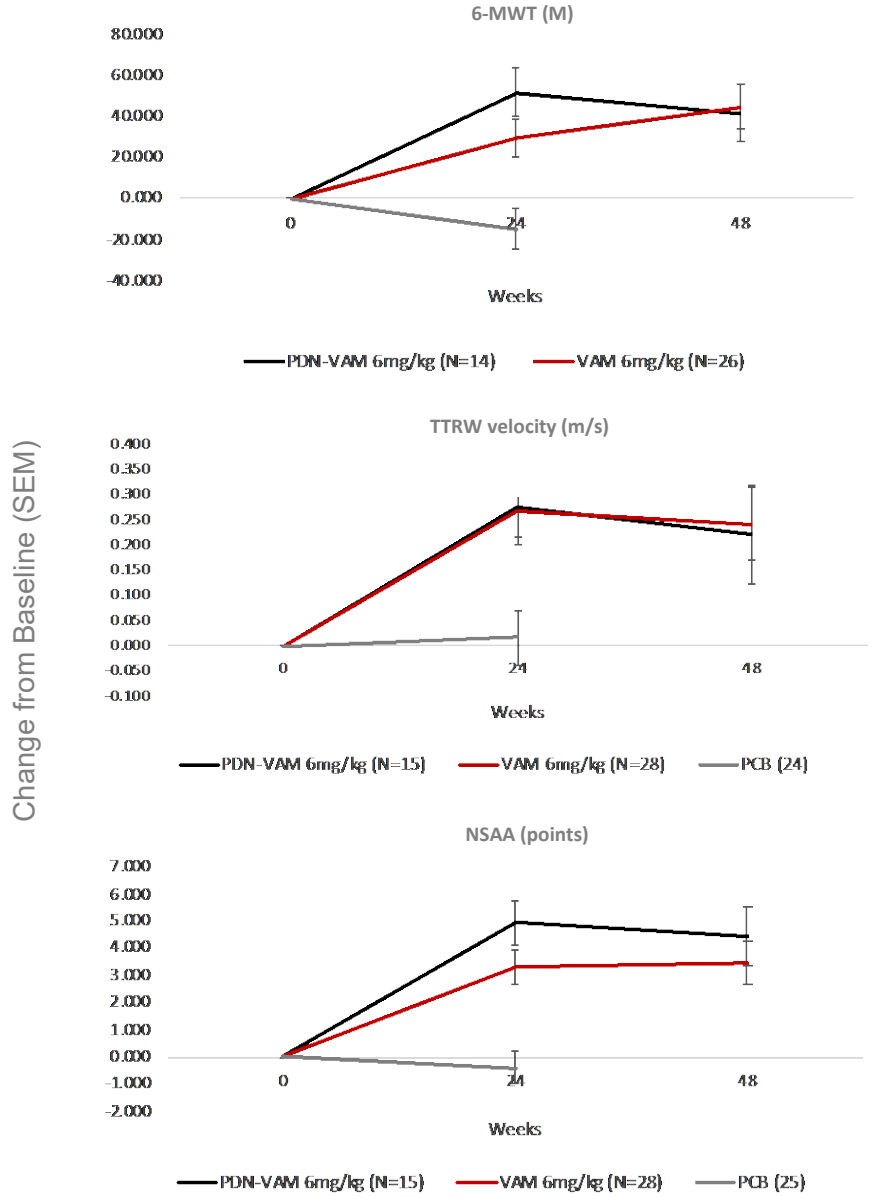
Boys who switched treatment from prednisone to vamorolone 6mg/kg maintained efficacy until week 48





PDN – Prednisone 0.75 mg/kg, All doses daily. MMRM estimates from mITT-2 population

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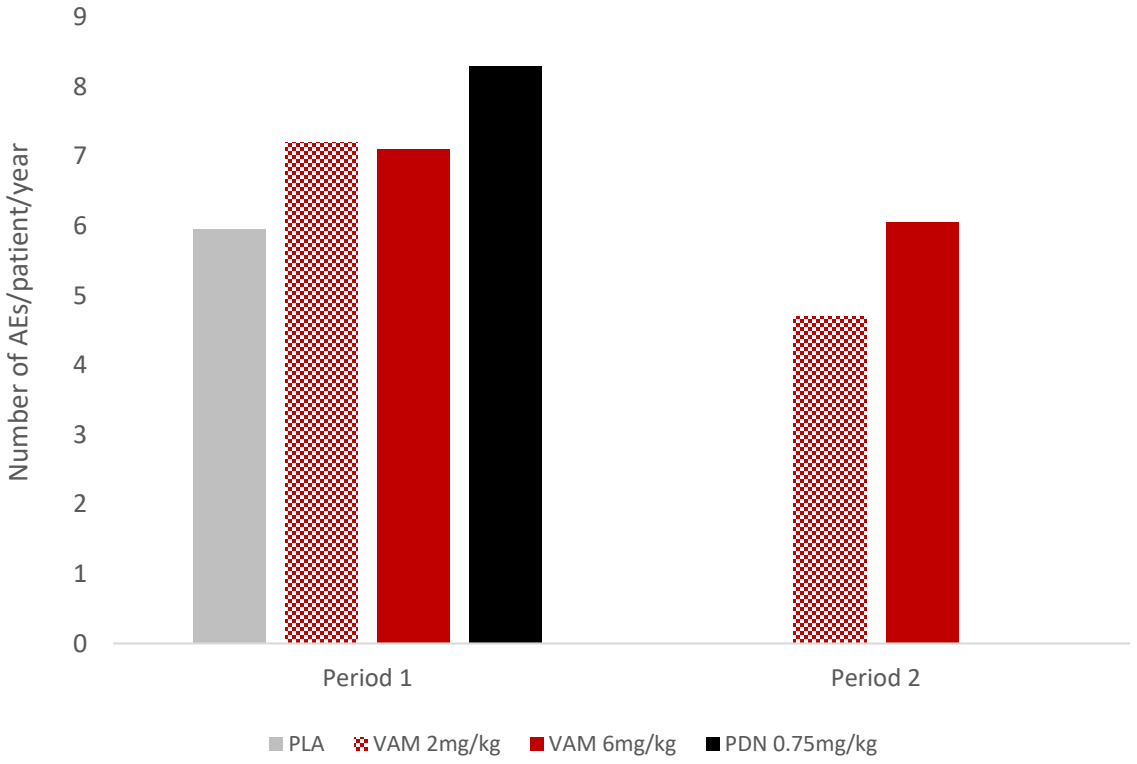
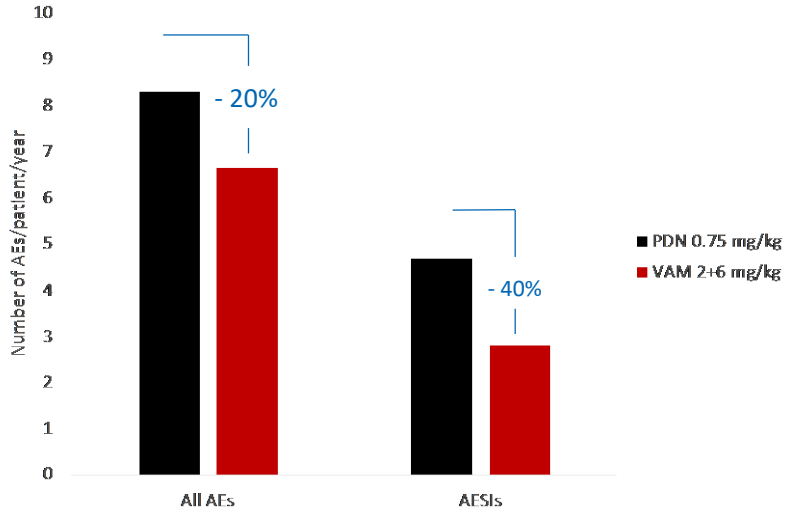
No increase in adverse events reported with prolonged vamorolone dosing



Vamorolone was generally safe & well tolerated

- 93% of subjects completed the entire 48-week study
- 3 SAEs unrelated to study drug
- 2 subjects discontinued treatment (1 withdrawal consent, 1 AE)
- Most common AEs reported were upper respiratory tract infection, vomiting, cushingoid

Fewer adverse events were reported in patients switching from prednisone to vamorolone



AE, adverse events; AESI, adverse events of special interest; SAE, serious adverse events. All doses daily. * Safety Population 2 (SAF-2)

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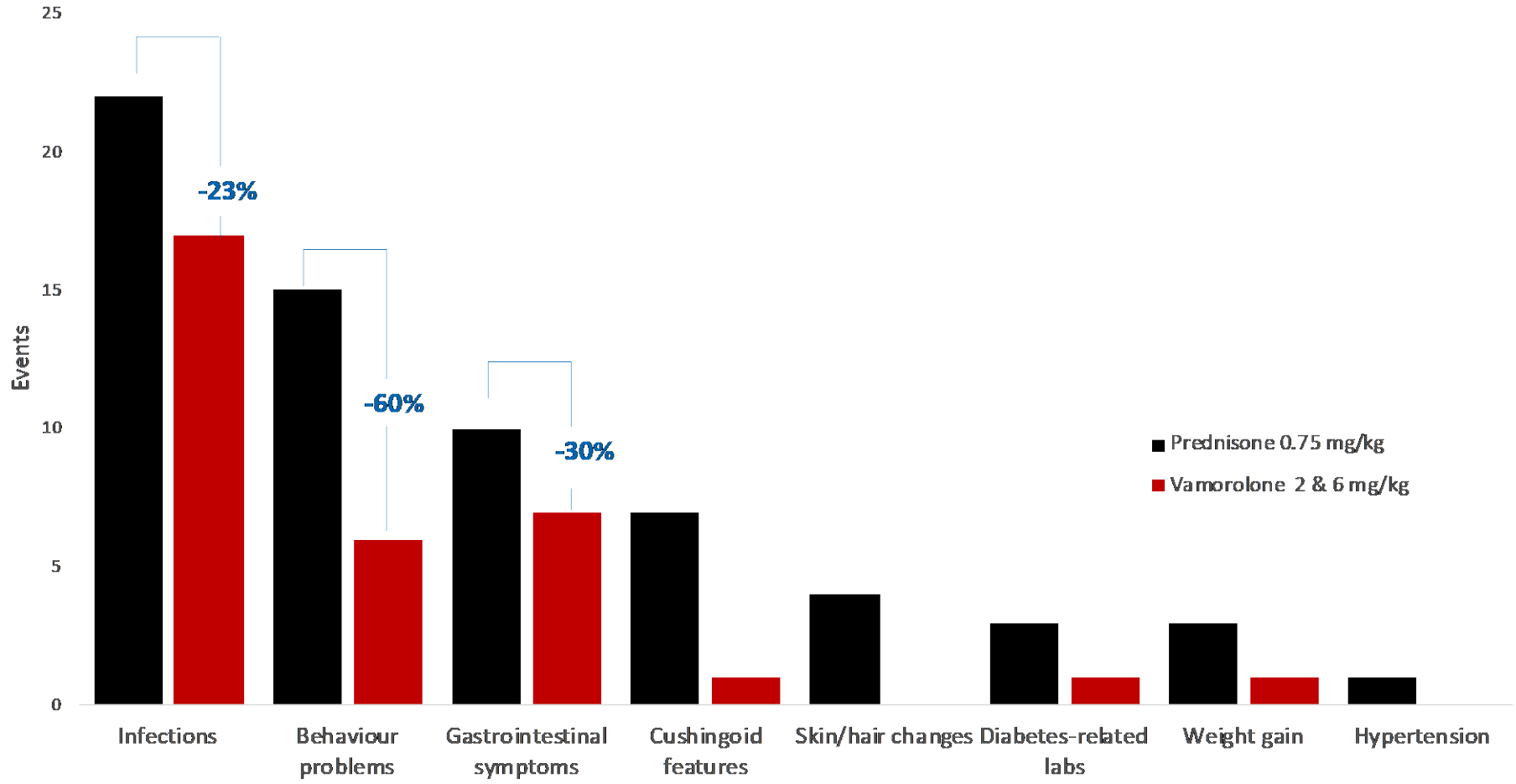
Physician's reported fewer AESI switching from prednisone to vamorolone

Comparison of Adverse Events of Special Interest (AESI)



Safety Population 2 (SAF-2), All doses daily

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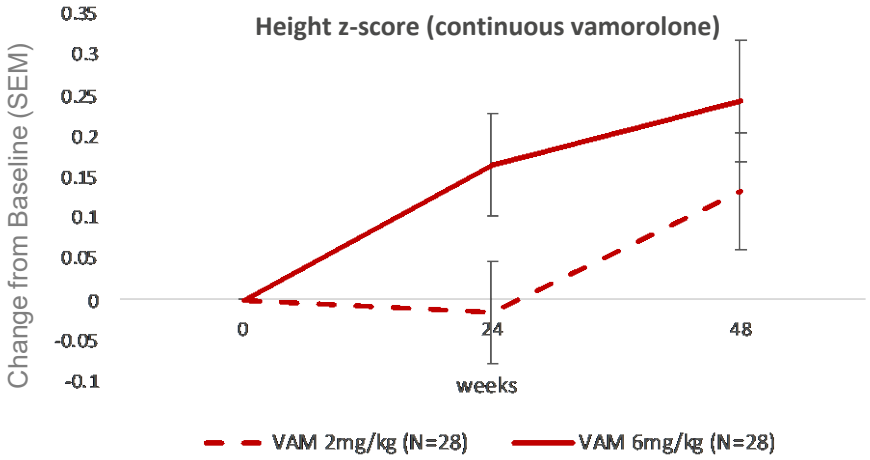
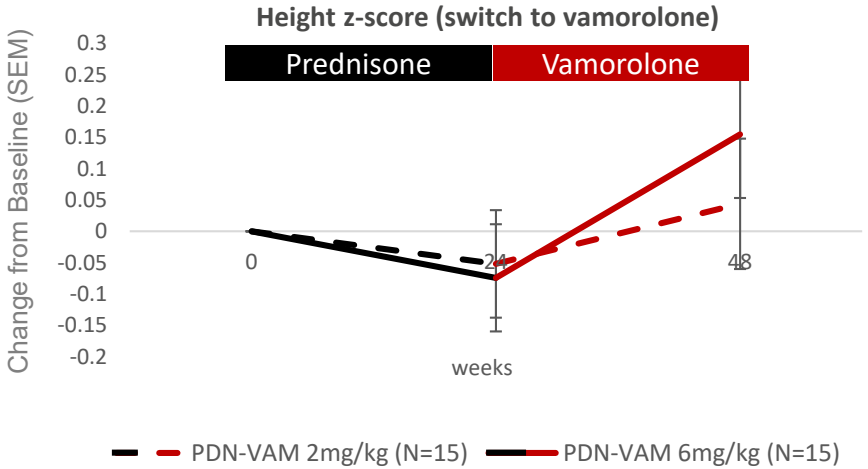
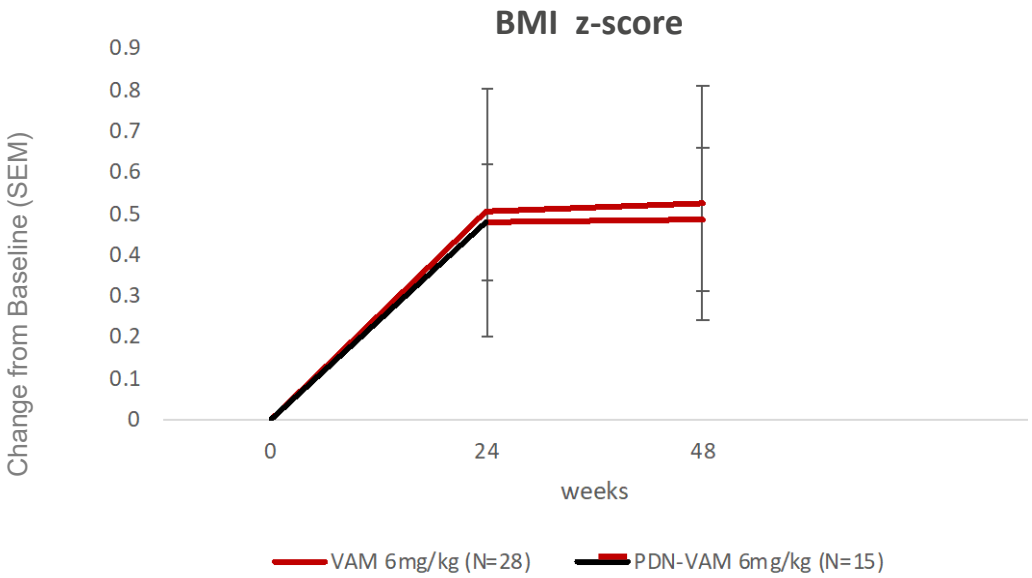
Boys continuously treated with vamorolone did not experience growth inhibition…



…and growth trajectory was restored in those switched from prednisone to vamorolone



BMI z-score remained stable for Period 2 for subjects both continuously dosed or switched to vamorolone



PDN – Prednisone 0.75 mg/kg, All doses daily, Safety Population 2 (SAF-2)

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Summary

- **Efficacy of vamorolone demonstrated at 24 weeks was maintained at 48 weeks with the 6mg/kg dose showing a more durable response compared to the 2mg/kg**

- **No increase in adverse events reported with prolonged use of vamorolone for either dose**

- **Switching from prednisone to vamorolone**
  - Did not result in a loss of efficacy over time with the 6mg/kg dose
  - Allowed boys to resume normal growth, experience fewer behavioral problems in addition to other side effects typically associated with steroid use

- **Vamorolone has demonstrated efficacy with a differentiated safety profile that could allow physicians to optimally treat patients for longer**

Q&A session

moderated by Laura Pfeifer-Rossi

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Closing

We thank you for your interest in Santhera

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