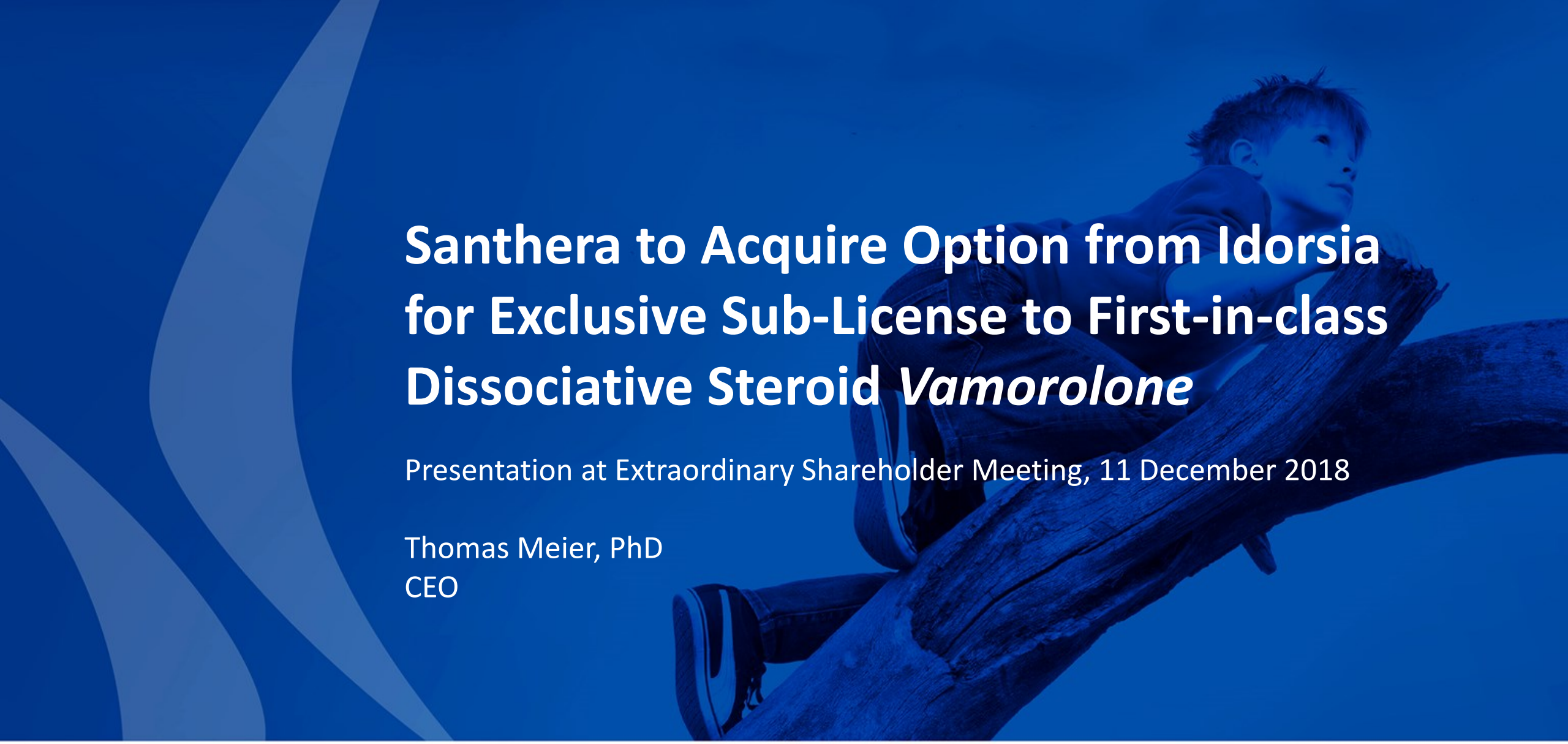

Santhera to Acquire Option from Idorsia for Exclusive Sub-License to First-in-class Dissociative Steroid *Vamorolone*

Presentation at Extraordinary Shareholder Meeting, 11 December 2018

Thomas Meier, PhD
CEO

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Vamorolone – a breakthrough treatment for patients with Duchenne muscular dystrophy (DMD)

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Vamorolone: a transformational opportunity for Santhera

- Glucocorticoids (GCs) are recognized standard of care in children and adolescent patients with DMD
- High-dose GCs have severe systemic side-effects preventing lifelong treatment
- Regulators and patients/families seek better tolerable alternatives to the current GCs (see below)
- ***Vamorolone* is a first-in-class therapy to replace existing GCs as a potential new standard of care for DMD**
- **ReveraGen**, the developer of *vamorolone*, granted Actelion followed by **Idorsia** a worldwide, unrestricted, exclusive option to license the product for commercialization. Under the agreement, Santhera will obtain an option to a sub-license for *vamorolone* in all indications worldwide excluding Japan and South Korea
- Basis for approval of *vamorolone* in DMD will be existing successful Phase IIa trial data and results from ongoing Phase IIb trial
- Time to approval targeted to be 2H 2021 in US and early 2022 in EU – Santhera estimates **market potential of USD 500m**
- Perfect strategic fit: *vamorolone* complements *idebenone* as treatment for DMD
- Idorsia largest shareholder in Santhera with a 13.3% equity stake (lock-up expiring when FDA approves *vamorolone* in the US)

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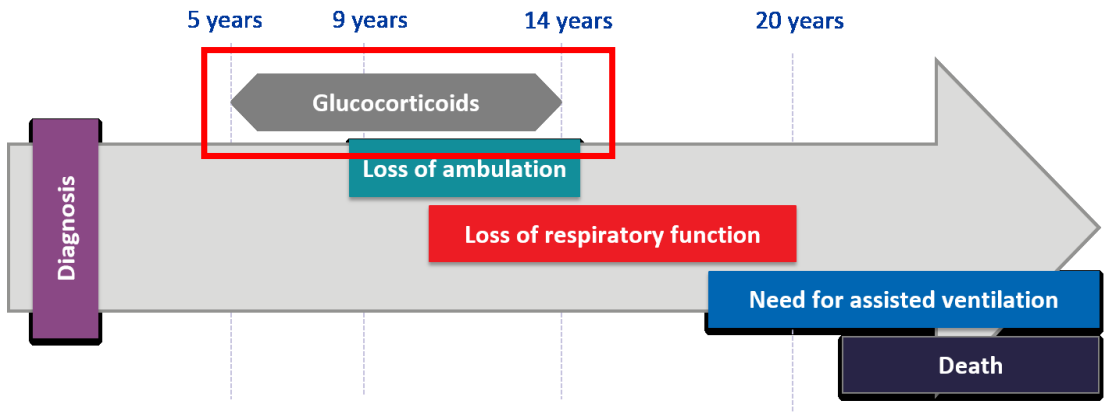
Urgent medical need for new treatments in DMD

- Glucocorticoids (GCs) are recognized standard of care in children and adolescent patients
- Therapeutic goal of GCs is to preserve upper and lower limb strength/function and ambulation
- EMFLAZA (*deflazacort*) approved and marketed in the US by PTC Therapeutics
- High-dose GCs have severe systemic side effects preventing lifelong treatment
- Regulators and patients/families seek better tolerable alternatives to the current GCs
- *Vamorolone* has the potential to become new standard of care replacing “old” glucocorticoids

Disease progression and clinical milestones in patients with DMD



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Vamorolone – discovered and developed by ReveraGen BioPharma

Eric Hoffman, PhD
President and CEO

John McCall, PhD
Chairman of the Board
Vice President of Chemistry

Kanneboyina Nagaraju, PhD
Treasurer, Secretary
Vice President of Research

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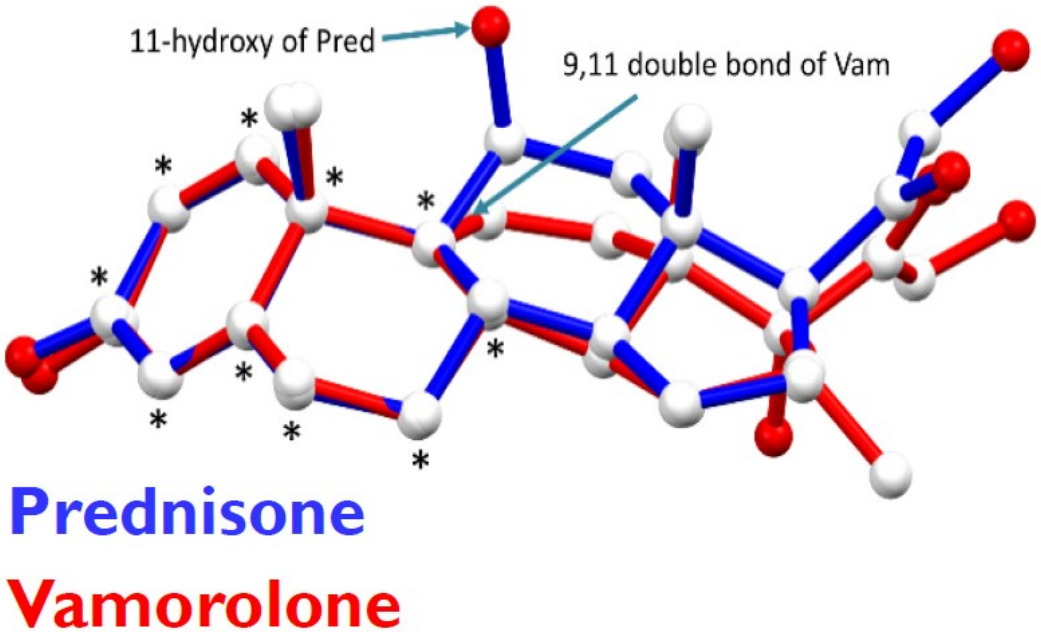
Vamorolone

- **First-in-class dissociative steroidal anti-inflammatory drug**
- Close analog to prednisone, a standard glucocorticoid (GC)
- Different pharmacological properties allow dissociation of beneficial effects from GC-class side effects



Source: www.reveragen.com

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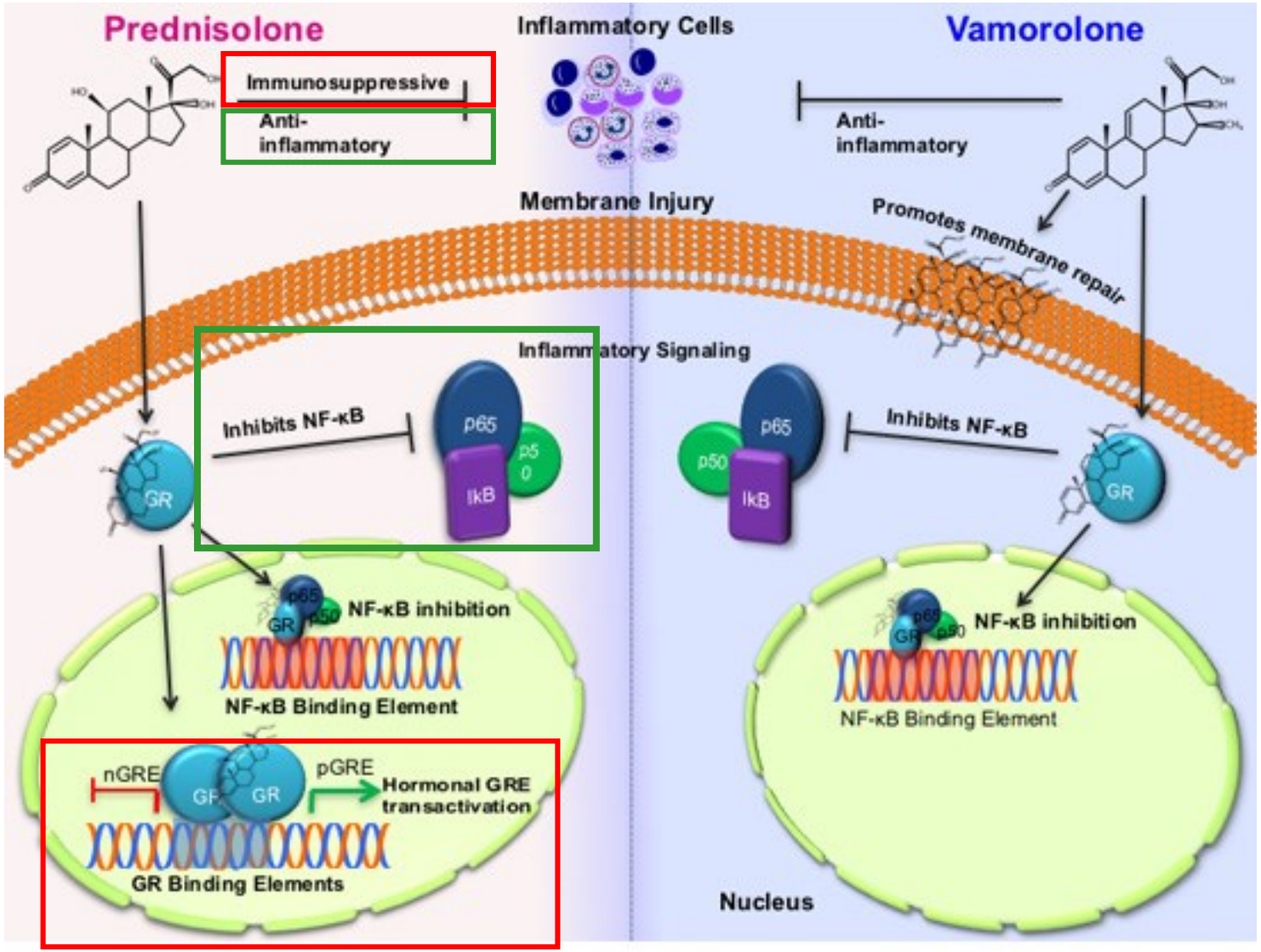
Vamorolone – revolutionary mode of action



Source: www.reveragen.com

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Vamorolone – current efficacy and safety data

Effects of *vamorolone* in animal model for DMD:

- Retains GC-type anti-inflammatory efficacy and reduces dystrophy, improves muscle strength and motor function
- Superior safety profile with respect to loss of stunting growth, bone symptoms, cardiac side effects

Effects of *vamorolone* in Phase I trial of healthy volunteers and Phase IIa trial in patients with DMD:

- *Vamorolone* was well tolerated at all dose levels (up to 20mg/kg/d)
- *Vamorolone* shows efficacy in patients with DMD comparable to standard glucocorticoids (GCs)
- Reduced GC-class side effects (weight gain, bone fragility, metabolic disturbance, immune suppression)

Phase 1 trial of vamorolone, a first-in-class steroid, shows improvements in side effects via biomarkers bridged to clinical outcomes

Eric P. Hoffman[a,b,*], Valerie Riddle[c], Maxime A. Siegler[d], Daniel Dickerson[e], Miroslav Backonja[e], William G. Kramer[f], Kanneboyina Nagaraju[a,b], Heather Gordish-Dressman[g], Jesse M. Damsker[a], John M. McCall[a]

Phase IIa trial in Duchenne muscular dystrophy shows vamorolone is a first-in-class dissociative steroidal anti-inflammatory drug

Laurie S. Conklin[a,b,1], Jesse M. Damsker[a,1], Eric P. Hoffman[a,c], William J. Jusko[d],

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Vamorolone – pivotal Phase IIb trial (VBP15-004), ongoing

The Vision-DMD trial	
Design	Phase IIb randomized, double-blind, parallel group, placebo- and active-controlled study with double-blind extension
Participants	**120 ambulant** boys ages **4 to <7 years, not taking steroids**
Randomization	1:1:1:1 randomization (*vamorolone* 2.0 mg/kg/day : *vamorolone* 6.0 mg/kg/day : prednisone 0.75 mg/kg/day : placebo)
Dosing	**Orally** at daily doses of **2.0 mg/kg** and **6.0 mg/kg** versus **prednisone** 0.75 mg/kg/day and placebo
Treatment	**24 week treatment period #1** (weeks 1-24), a 4-week transition period (weeks 25-28), a **20-week treatment period #2** (weeks 28-48), and a 4-week dose-tapering period (weeks 49-52); one visit per month
Protocol	Developed under FDA and EMA scientific advice; “pivotal” trial
Study start/end	Start: August 2018; estimated end: 2H 2020
Primary outcome	Muscle function measured by **Time to Stand Test** Body weight as measured by body mass index (**BMI**) z-score
Secondary outcomes	Safety, cardiac function, efficacy: 6MWT, NSAA, run/walk test; other secondary outcome measures
Study conduct	Approximately 30 sites in US (recruiting), EU, Canada, Australia, Israel

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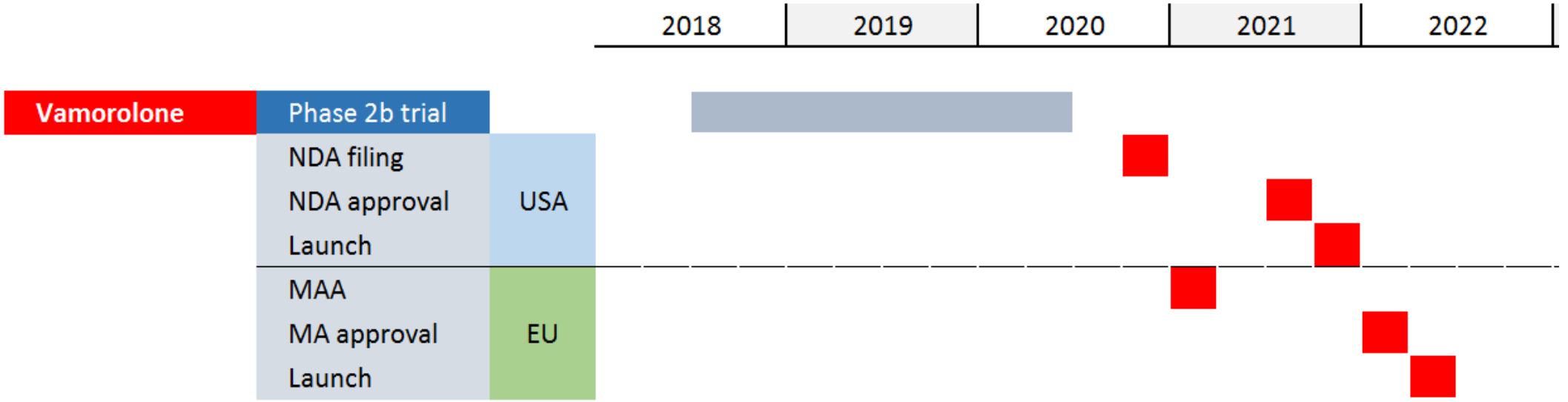
Estimated time to market, protection and sales potential



NDA: new drug application; MAA: marketing authorization application

Protection and Regulatory Status

- Orphan drug protection: USA (7y) and EU (10y)
- Method of use patent until 2029 (by country)
- Fast-track designation in USA

Competitive Positioning and Sales Potential

- *Vamorolone* to become standard of care
- Efficacy comparable/superior to standard GCs avoiding severe side effects
- Sales potential of USD 500m

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Excellent strategic fit – *Vamorolone* complements Santhera's DMD program and strengthens its pipeline

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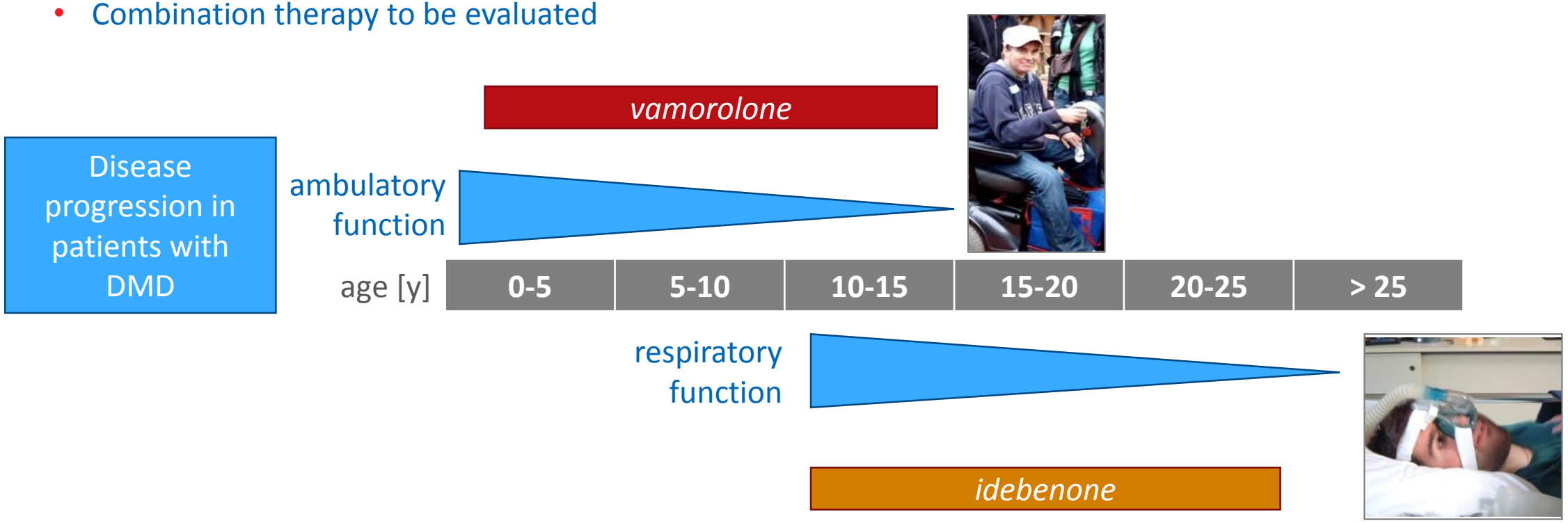
Pipeline synergies between *vamorolone* and *idebenone*

- Combination of *vamorolone* and *idebenone* addresses medical need of DMD patients at all disease stages
- *Vamorolone* and *idebenone* could be used in all patients (not restricted to certain mutations)
- Combination therapy to be evaluated



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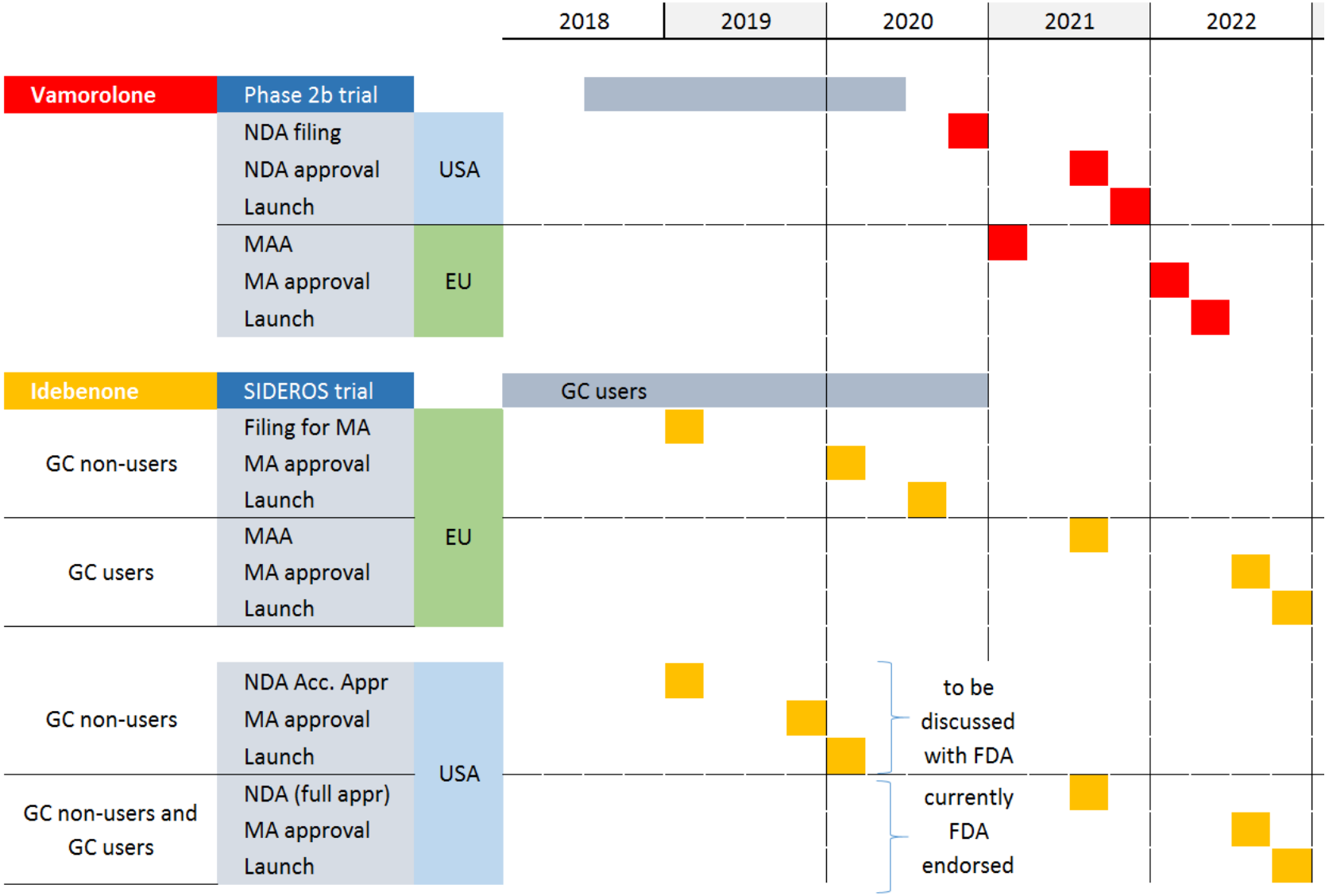
Targeted time to market for Santhera's DMD franchise



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Santhera's *pro forma* pipeline post-transaction

Santhera Pipeline	Drug	Preclin.	Phase I	PoC	Pivotal	Filing	Market
Neuro-ophthalmological diseases							
Leber's hereditary optic neuropathy	Idebenone						*Raxone®*
Neuromuscular diseases							
Duchenne muscular dystrophy (GC non- users)	Idebenone				completed		
Duchenne muscular dystrophy (GC users)	Idebenone				ongoing		
Duchenne muscular dystrophy	Vamorolone				ongoing	ReveraGen BioPharma	
Congenital muscular dystrophy	Omigapil		completed				
Pulmonary diseases							
Cystic fibrosis	POL6014		start Q4-18				
AAT, NCFB and PCD	POL6014		to be explored				
Chronic obstructive pulmonary disease	POL6014						

GC: Glucocorticoid; AAT: Alpha-1 antitrypsin deficiency; NCFB: Non-cystic fibrosis bronchiectasis; PCD: primary ciliary dyskinesia; PoC: proof of concept

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Thank you for your attention

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