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SRK-015 Interim Phase 1 Results and Phase 2 Overview

February 26, 2019

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Agenda for SRK-015 Phase 1 and Phase 2 Call

- **Introductory Remarks** – Nagesh Mahanthappa, *President and Chief Executive Officer*
- **Phase 1 Interim Results** – Yung Chyung, *Chief Medical Officer*
- **Rationale for Investigating SRK-015 in SMA** – Yung Chyung, *Chief Medical Officer*
- **Overview of the Phase 2 Trial** – Yung Chyung, *Chief Medical Officer*
- **Key Milestones and Closing Remarks** – Nagesh Mahanthappa, *President and CEO*
- **Q&A**

Robust Pipeline Portfolio

Program / Target		Indication	Discovery / Early Preclinical	Preclinical	Phase 1	Phase 2	Rights / Partner	Next Anticipated Milestone
INTERNAL PROPRIETARY PROGRAMS								
SRK-015	Pro/Latent Myostatin	Spinal Muscular Atrophy						Initiate Phase 2 POC Trial in 1Q19
	Pro/Latent Myostatin	Myostatin-Related Disorders						1H19 – Identify Next Indication
Latent TGFβ	Context-Independent Latent TGFβ1	Oncology/Immuno-oncology						1H19 – Nominate Product Candidate
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	Undisclosed Program	Fibrosis					GILEAD	
	Context-Dependent Latent TGFβ1 / GARP	Oncology/Immuno-oncology					*Janssen Biotech, Inc*	

2018: Transformative Year for Scholar Rock

Established Strong Financial Foundation

- Transitioned to public company with successful $86M IPO

Transitioned to Clinical-Stage Company

- SRK-015 Phase 1 trial supports advancement to Phase 2 SMA proof-of-concept trial

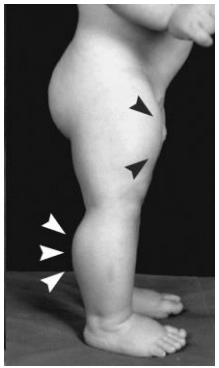
Executed Strategic Collaboration

- Gilead fibrosis collaboration with $80M upfront and up to $1.45B in milestones

Advanced Innovative Pipeline

- Progressed antibody platform for neuromuscular disorders, cancer immunotherapy, fibrosis, and anemias

SRK-015: Highly Specific Inhibitor of Latent Myostatin



- Myostatin is a genetically-validated, negative regulator of muscle mass expressed in skeletal muscle tissue
- Vertebrates lacking the myostatin gene are healthy and display increased muscle mass and strength

Differentiated approach with SRK-015:

- ***Fully human monoclonal antibody (mAb)***
- ***Highly selective inhibitor of the activation of myostatin precursor***
- ***Half-life of 23-33 days***
- ***Orphan Drug Designation for SMA granted by FDA and EC***
- ***US Patent 9,758,576 covers mAbs that inhibit the activation of the myostatin precursor (expiry in 2034)***

SRK-015: Positive Phase 1 Interim Results

SRK-015 Achieved Key Objectives of Phase 1 Trial:

- **Well-tolerated with no apparent safety signals**
  - No dose-limiting toxicities identified up to highest evaluated dose of 30 mg/kg
  - No discontinuations or SAEs related to SRK-015, or hypersensitivity reactions
- **Half-life of 23-33 days supports planned evaluation of Q4W dosing in Phase 2 trial**
- **Robust target engagement provides proof-of-mechanism**
  - Marked increases in serum latent myostatin levels
  - Evidence for target saturation starting as low as 3 mg/kg dose
- **Supports advancing to Phase 2 trial by the end of 1Q19**

Highly specific inhibitor of the activation of myostatin

First step towards validating Scholar Rock's unique mechanism of action and platform

Phase 1 Interim Results

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SRK-015 Phase 1 Trial Design

KEY OBJECTIVES OF PHASE 1

Evaluate the safety and tolerability, pharmacokinetics, and pharmacodynamics of SRK-015 IV

	SINGLE-ASCENDING DOSE (SAD)	MULTIPLE-ASCENDING DOSE (MAD)
Design	Double-blind, placebo-controlled 3:1 randomization	Double-blind, placebo-controlled 3:1 randomization
Subjects	40 Adult healthy volunteers (Ages 18-55)	26 Adult healthy volunteers (Ages 18-55)
Dosing	Single doses at: 1, 3, 10, 20, or 30 mg/kg	Q2W dosing for 3 doses at: 10, 20, or 30 mg/kg

Phase 1 Interim Safety Results Support Advancing to Phase 2 Trial

- **SRK-015 was well-tolerated with no apparent safety signals**
- **No dose-limiting toxicities identified up to highest evaluated dose of 30 mg/kg**
  - No discontinuations due to a treatment-related AE
  - No treatment-related SAEs or deaths
  - No hypersensitivity reactions
- **Anti-drug antibody tests were negative in SAD; MAD data pending**
- **SAD cohort: AEs* were observed in 30% (9/30) SRK-015- vs. 50% (5/10) placebo-treated subjects**
  - Most frequently reported AE: headache
- **MAD** cohort: AEs observed in 30% (6/20) SRK-015- vs. 67% (4/6) placebo-treated subjects**
  - Most frequently reported AE: postural dizziness
- **Single reported SAE of gallstone-induced pancreatitis**
  - Assessed by trial investigator as unrelated to SRK-015 treatment

*The term "adverse event" noted in this presentation refers to a treatment-emergent adverse event, which is defined as an AE with onset after administration of study drug through the final follow-up visit, or in the event that onset time precedes study drug administration, the AE increases in severity during the post-dosing follow-up period
** MAD analysis includes data to interim cut-off (Day 35 for 30 mg/kg dose cohort and longer follow-up for 10 and 20 mg/kg dose cohorts)

PK Data Support Infrequent Dosing

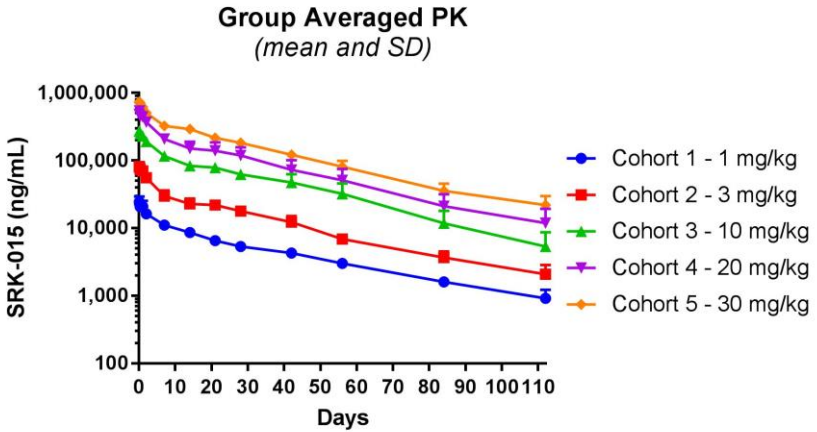
Displayed Well-Behaved PK Profile

- Consistent with that commonly observed with monoclonal antibodies
- Dose-proportional serum drug exposure

Half-Life Supports Infrequent Dosing

- Serum half-life of 23-33 days across the SRK-015 dose groups
- Supports planned evaluation of once every 4-week (Q4W) dosing in Phase 2

Pharmacokinetic (PK) Data from Single-Ascending Dose



PD Data Demonstrate Robust and Sustained Target Engagement

Robust Target Engagement Observed

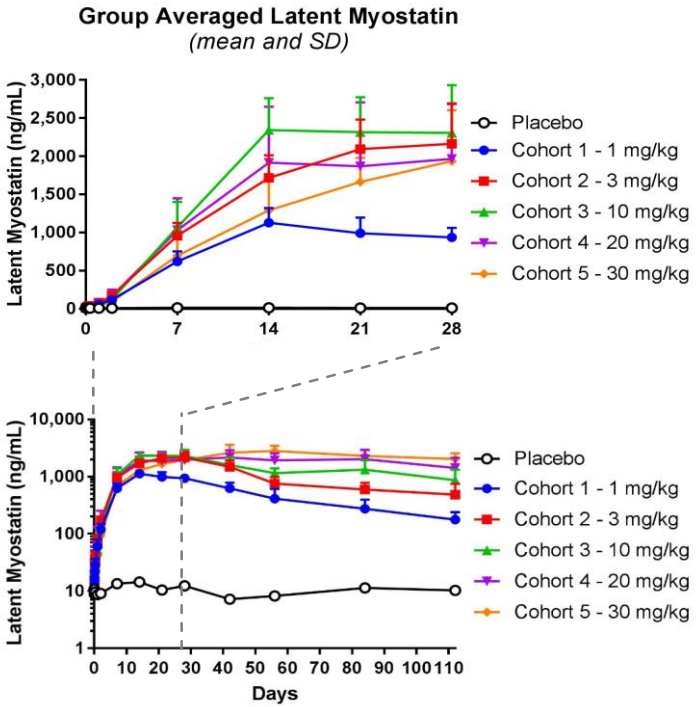
- Marked increases in serum concentrations of latent myostatin following a single dose of SRK-015
- No meaningful change observed with placebo

Evidence Supports Durable Target Saturation

- Peak latent myostatin levels plateaued starting with a single dose at 3 mg/kg suggesting target saturation
  - Single dose at 1 mg/kg only attained approximately half of the peak level
- Plateau was sustained demonstrating durability of effect:
  - Thru Day 28 after single dose at 10 mg/kg dose
  - Thru at least Day 84 after single doses at 20 and 30 mg/kg

Initial proof-of-mechanism in humans of Scholar Rock's therapeutic approach targeting the latent form of growth factors

Biomarker/Pharmacodynamic (PD) Data from Single-Ascending Dose



Rationale for Investigating SRK-015 in SMA

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Significant Unmet Need Remains Despite Current Therapies

SMN Upregulator Therapies

Address SMN deficiency to prevent further motor neuron deterioration

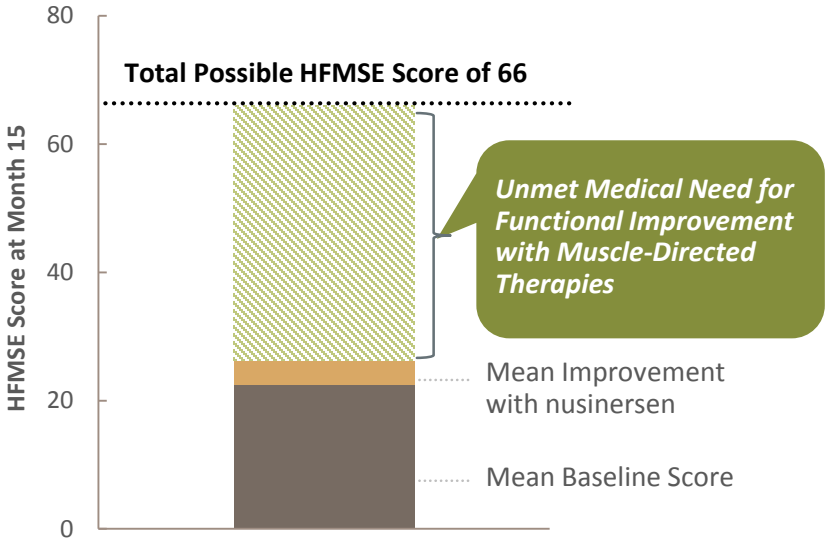
Muscle-Directed Therapies (SRK-015)

Act directly on muscle with aim to improve functional performance

SRK-015 has the potential to drive functional performance across a range of severity observed in SMA either as a monotherapy or in conjunction with any SMN upregulator/corrector therapy

Muscle Function in SMA (Human)

Hammersmith Functional Motor Scale Expanded (HFMSE)

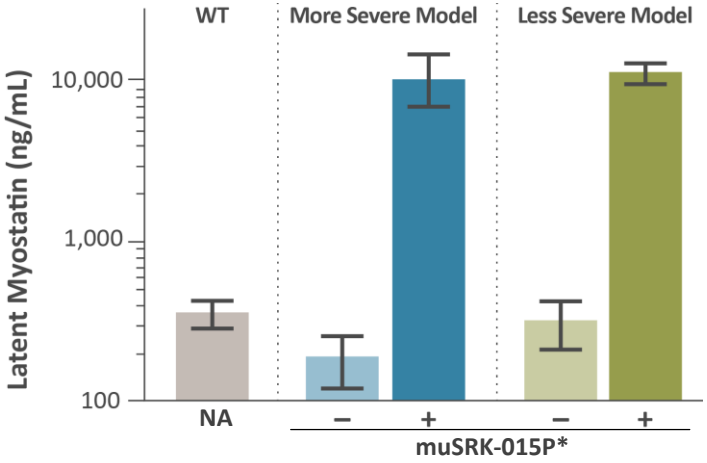


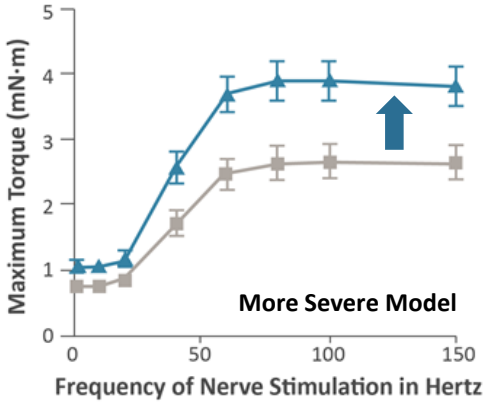
Mean improvement in HFMSE score experienced by patients with later-onset SMA in the Phase 3 CHERISH clinical trial of nusinersen

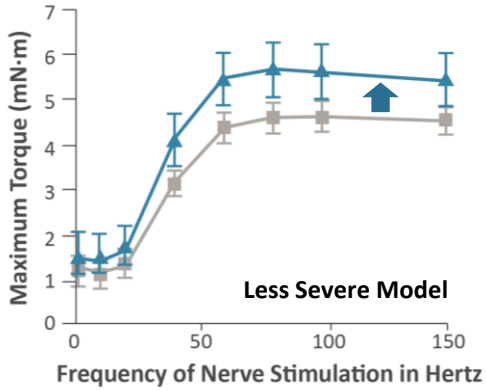
SRK-015: Aligning Therapeutic Approach with Myostatin Biology

Scholar Rock's Guiding Principles for Neuromuscular Indication Selection	Key Characteristics of Spinal Muscular Atrophy (SMA)
Younger population	Genetic disorder with onset in childhood
At least partially intact innervation and no structural muscle abnormalities	Partial neural connectivity and atrophied muscles that largely retain structural integrity
Need for increase in fast-twitch muscle fibers	Substantial deficit in fast-twitch fibers
Clinical trial endpoint driven by fast-twitch fiber function	Fast-twitch fiber function has a prominent role in SMA outcome measures

Review of Preclinical Data in SMNΔ7 Mouse Models







- **Achieved multi-fold increase in serum latent myostatin levels indicating target engagement**
- **Confirms presence of target in disease setting**
- **Lower latent myostatin levels in the SMA group may be attributable to reduced overall muscle mass**

Treatment improved muscle mass and strength

- Maximal torque of the plantar flexor muscle group increased:
  - More severe model: 44%-51%
  - Less severe model: 20%-30%

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*muSRK-015P is a murine version of SRK-015

Preclinical data published Nov 2018: Long, K., O'Shea, K., Khairallah, R., et al. Specific Inhibition of Myostatin Activation is Beneficial in Mouse Models of SMA Therapy. *Human Molecular Genetics,* ddy382

SRK-015 Target Profile in SMA

GOALS	EVIDENCE TO DATE
Effectively increase motor function to drive clinically meaningful outcomes	✓ Translational/preclinical data support myostatin as a drug target in SMA ✓ Preclinical data demonstrate potential for substantial increases in muscle strength ✓ Interim Phase 1 PD data demonstrate SRK-015 can successfully engage the target in a durable fashion
Safety profile to enable chronic dosing, including in pediatric populations	✓ Well-tolerated with no apparent safety signals based on Phase 1 interim data ✓ Binds myostatin precursors with high selectivity in vitro
Low drug administration burden to offer broad accessibility	✓ Minimally invasive route of administration (IV) ✓ Interim PK and PD data support an infrequent dosing regimen (e.g. once every 4 weeks)

Emerging evidence supports investigating the safety and efficacy of SRK-015 in SMA

Phase 2 SMA Trial Overview

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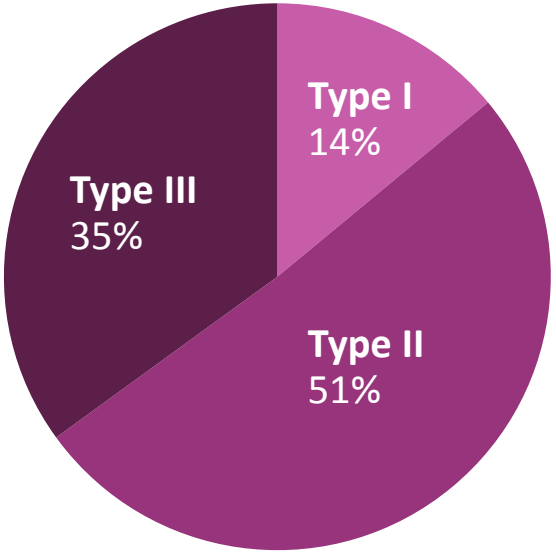
SRK-015 Opportunity in Spinal Muscular Atrophy

Overall Prevalence of 30,000-35,000 in U.S. and Europe

Relative Prevalence Among Patients Living With SMA



Type I:

- Infant-onset; often fatal

Type II and non-ambulatory type III:

- Later-onset but still early childhood
- Severe deficits in motor function

Ambulatory type III:

- Limited mobility and substantial morbidity

Type IV:

- Population not well-characterized

Focus of Phase 2 Trial

Potential to use SRK-015 in conjunction with SMN upregulators

Potential to use SRK-015 as monotherapy or in conjunction with SMN upregulators

Lally et al, Orphanet Journal of Rare Diseases, 2017

Overview of Phase 2 TOPAZ trial in SMA

Design

- 3 cohorts; total of 50-60 patients
- 12-month treatment period
- SRK-015 IV every 4 weeks (Q4W) as monotherapy or in conjunction with an approved SMN upregulator therapy

Subjects

- Type 2 and Type 3 SMA
- Each of the 3 cohorts will encompass a distinct subpopulation of patients

Key Objectives

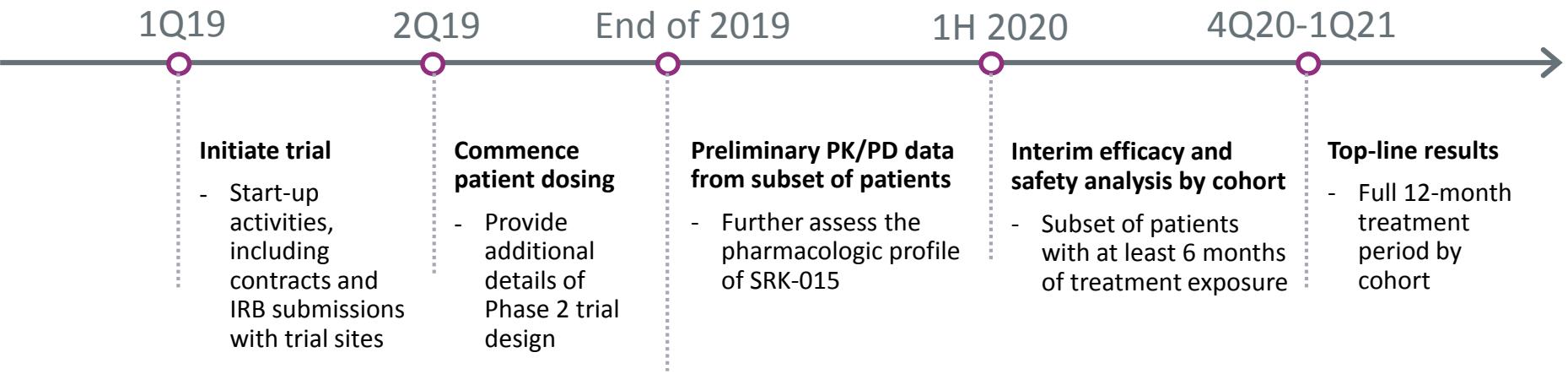
- HFMSE (non-ambulatory SMA)
- RHS (ambulatory SMA)
- Safety

Timeline

- Preliminary PK/PD by end of 2019
- Interim efficacy/safety analysis in 1H20
- Top-line results 4Q20-1Q21

HFMSE – Hammersmith Functional Motor Scale Expanded; RHS – Revised Hammersmith Scale

SRK-015: Path to Top-Line Results in SMA



SRK-015 has the potential to be the first muscle-directed therapy for patients with SMA

Significant Milestones Ahead

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Key R&D Milestones

SRK-015

- Initiate Phase 2 SMA proof-of-concept trial by the end of 1Q19
- Commence patient dosing in Phase 2 SMA proof-of-concept trial in 2Q19
- Present full Phase 1 results at a scientific conference in 2019
- Continue to evaluate selective inhibitors of myostatin activation in multiple disease models
- Identify next indication in 1H19
- Announce Phase 2 trial read-outs:
  - Preliminary PK/PD analysis by end of 2019
  - Interim efficacy and safety analysis at 6 months in 1H20
  - Top-line results of 12-month treatment period 4Q20-1Q21

TGFβ1 Inhibitor

- Continue to evaluate selective inhibitors of TGFβ1 activation in cancer immunotherapy models
- Nominate product candidate in TGFβ1 program in 1H19
- Continue to advance active discovery programs for context-dependent inhibition of TGFβ1
- Conduct fibrosis discovery and preclinical studies in partnership with Gilead

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Building Value in All Dimensions

Leveraging Strong Financial Foundation

Advancing Clinical Development

Executing Strategic Collaboration

Growing Innovative Pipeline