UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 8-K

CURRENT REPORT

Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

Date of Report (Date of earliest event Reported): November 19, 2019

Scholar Rock Holding Corporation
(Exact Name of Registrant as Specified in Charter)

Delaware	**001-38501**	**82-3750435**
(State or Other Jurisdiction of Incorporation)	(Commission File Number)	(I.R.S. Employer Identification Number)

620 Memorial Drive, 2nd Floor, Cambridge, MA 02139
(Address of Principal Executive Offices) (Zip Code)

(857) 259-3860
(Registrant's telephone number, including area code)

(Former name or former address, if changed since last report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

- ☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- ☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- ☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- ☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	**Trading Symbol(s)**	**Name of each exchange on which registered**
Common Stock, par value $0.001 per share	SRRK	Nasdaq Global Select Market

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (17 CFR §230.405) or Rule 12b-2 of the Securities Exchange Act of 1934 (17 CFR §240.12b-2). Emerging growth company ☒

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☒

Item 7.01. Other Events.

The management of Scholar Rock Holding Corporation (the "Company") will participate in the Jeffries London Healthcare Conference in London, England on Wednesday, November 20, 2019 with a presentation at 1 p.m. ET.

A copy of the presentation slide deck that will be presented at the Jeffries London Healthcare Conference is being furnished as Exhibit 99.1 to this Report on Form 8-K. A live webcast of the presentation may be accessed by visiting the Investors & Media section of the Scholar Rock website at http://investors.scholarrock.com. The information in this Report on Form 8-K and Exhibit 99.1 attached hereto is intended to be furnished and shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such filing.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits

Exhibit No.	Description
99.1	Presentation Slide Deck, furnished hereto.

SIGNATURE

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Scholar Rock Holding Corporation

Date: November 19, 2019

By: /s/ Junlin Ho
Junlin Ho
Vice President, Head of Corporate Legal

SCHOLAR ROCK

From New Insights to New Medicines

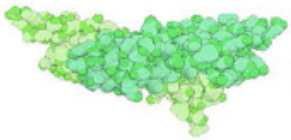
November 2019

Disclaimers

Various statements in this presentation concerning Scholar Rock's future expectations, plans and prospects, including without limitation, Scholar Rock's expectations regarding its strategy, its product candidate selection and development timing, including timing for the initiation of and reporting results from its clinical trials for its product candidates, its disease indication selection and timing for such selection, the ability of SRK-015 to affect the treatment of patients suffering from Spinal Muscular Atrophy (SMA) either as a monotherapy or in conjunction with the current standard of care, the ability of SRK-181 to affect the treatment of cancer patients in a manner consistent with preclinical data, and the projected use of cash constitute forward-looking statements for the purposes of the safe harbor provisions under The Private Securities Litigation Reform Act of 1995. The use of words such as "may," "might," "will," "should," "expect," "plan," "anticipate," "believe," "estimate," "target," "project," "intend," "future," "potential," or "continue," and other similar expressions are intended to identify such forward looking statements. Actual results may differ materially from those indicated by these forward-looking statements as a result of various important factors, including, without limitation, Scholar Rock's ability to provide the financial support and resources necessary to identify and develop multiple product candidates on the expected timeline, competition from others developing products for similar uses, the preliminary nature of interim clinical data, Scholar Rock's ability to obtain, maintain and protect its intellectual property, Scholar Rock's dependence on third parties for development and manufacture of product candidates including to supply any clinical trials, and Scholar Rock's ability to manage expenses and to obtain additional funding when needed to support its business activities and establish and maintain strategic business alliances and new business initiatives as well as those risks more fully discussed in the section entitled "Risk Factors" in the Quarterly Report on Form 10-Q for the quarter ended September 30, 2019, which is on file with the Securities and Exchange Commission, as well as discussions of potential risks, uncertainties, and other important factors in Scholar Rock's subsequent filings with the Securities and Exchange Commission. Any forward-looking statements represent Scholar Rock's views only as of today and should not be relied upon as representing its views as of any subsequent date. Scholar Rock explicitly disclaims any obligation to update any forward-looking statements unless required by law.

Novel Approach to Selective Modulation of Growth Factor Signaling

*Traditional approaches inhibit growth factors **after** activation*



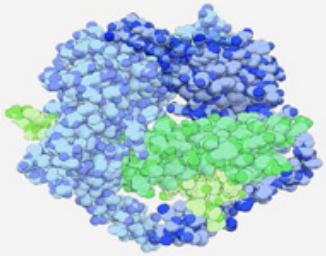
Traditional Target

"Mature" Growth Factor

...but have been limited by:

- Structural similarities
- Overlapping sets of related receptors
- Diverse and overlapping physiological roles

Scholar Rock's Target



Growth Factor Precursor (Latent Form)

- Inhibits **activation of growth factor precursors** to significantly improve:
  - Selectivity
  - Potency
  - Localization of effect
- Preclinical/clinical data demonstrate successful targeting of latent growth factors in disease settings
- Developing growing pipeline across neuromuscular disorders, oncology, fibrosis and anemia

Robust Pipeline Portfolio

	Target / Program	Indication	Discovery / Early Preclinical	Preclinical	Phase 1	Phase 2	Rights / Partner	Next Anticipated Milestone
INTERNAL PROPRIETARY PROGRAMS								
Pro/Latent Myostatin	SRK-015	Spinal Muscular Atrophy (3 distinct Type 2 and Type 3 patient populations)						Interim Efficacy and Safety Results in 1H20
	SRK-015	Myostatin-Related Disorders						Identify Next Indication in 2020
Latent TGFβ	SRK-181 (Context-Independent Latent TGFβ1)	Immuno-oncology (Primary resistance to CBTs*)						Initiate Phase 1 Trial in Patients with Solid Tumors in 1Q20
	SRK-181 (Context-Independent Latent TGFβ1)	Oncology						
	Context-Dependent Latent TGFβ1 / Immune Cell	Oncology/Immuno-oncology						
RGMc	BMP6 Signaling Pathway (anti-RGMc)	Iron-Restricted Anemias						Nominate Product Candidate in 2020
PARTNERED PROGRAMS								
Latent TGFβ	Context-Independent Latent TGFβ1	Fibrosis					GILEAD	
	Context-Dependent Latent TGFβ1 / LTBP1 & LTBP3	Fibrosis					GILEAD	
	Undisclosed Program	Fibrosis					GILEAD	
	Context-Dependent Latent TGFβ1 / GARP	Oncology/Immuno-oncology					Janssen Biotech, Inc	

*CBTs = checkpoint blockade therapies

Upcoming Key R&D Milestones

Spinal Muscular Atrophy (SMA)

- ✓ Initiate SRK-015 Phase 2 proof-of-concept SMA trial by the end of 1Q19
- ✓ Commence patient dosing in SRK-015 Phase 2 SMA proof-of-concept trial in 2Q19
- ✓ Present final Phase 1 results at Cure SMA Annual Conference being held June 28-July 1, 2019
- • Announce SRK-015 Phase 2 trial read-outs:
  - ✓ Preliminary PK/PD analysis by end of 2019
  - ○ Interim efficacy and safety results in a subset of patients at 6 months in 1H20
  - ○ Top-line results of 12-month treatment period 4Q20-1Q21

Oncology

- ○ Initiate SRK-181 Phase 1 dose escalation and POC trial in patients with solid tumors in 1Q20
- ○ Continue to advance active discovery programs for context-dependent inhibition of TGFβ1

Fibrosis

- ○ Continue to conduct fibrosis discovery and preclinical studies in partnership with Gilead

Anemias

- ○ Nominate product candidate in RGMc program in 2020

SRK-015: Inhibitor of Myostatin Activation

Potential First Muscle-Directed Therapy for SMA

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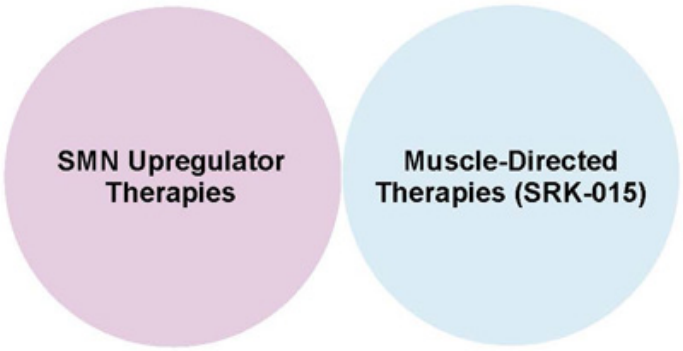
SRK-015: Highly Specific Inhibitor of Latent Myostatin

- 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
- Preclinical and clinical data to date provide strong rationale for developing SRK-015 to improve muscle function in Spinal Muscular Atrophy (SMA)

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)***

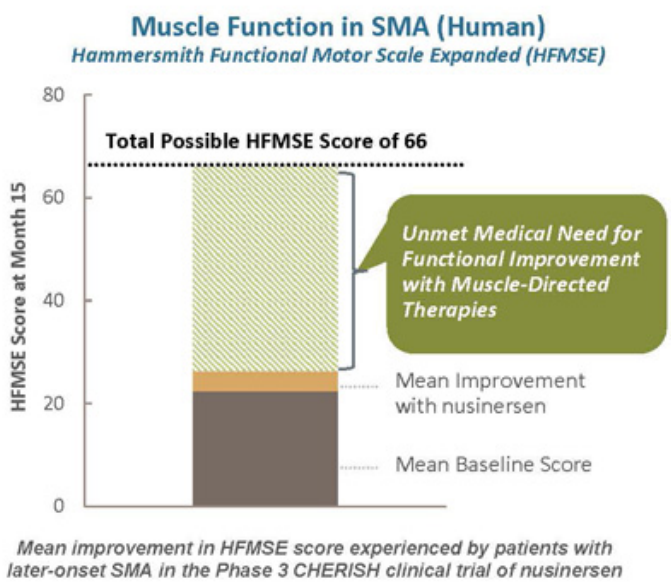
Significant Unmet Need Remains Despite Current Therapies



Address SMN deficiency to prevent further motor neuron deterioration

Act directly on muscle with aim to improve functional performance

SRK-015 has potential to drive functional performance across a range of severity observed in SMA



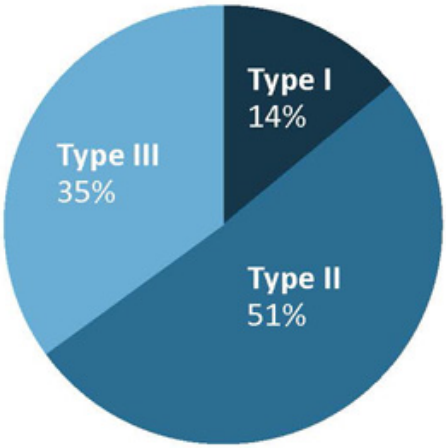
Mean improvement in HFMSE score experienced by patients with later-onset SMA in the Phase 3 CHERISH clinical trial of nusinersen

 SMN = survival motor neuron

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

SRK-015 Phase 2 Trial Design

TOPAZ

	Cohort 1	Cohort 2	Cohort 3
Design	• N= 20; ages 5-21 • Open-label, single-arm • 20 mg/kg SRK-015 IV Q4W • 12-month treatment period	• N= 15; ages 5-21 • Open-label, single-arm • 20 mg/kg SRK-015 IV Q4W • 12-month treatment period	• N= 20; ages ≥2 • Double-blind, randomized (1:1) to 2 mg/kg or 20 mg/kg SRK-015 IV Q4W • 12-month treatment period
Subjects	• Ambulatory Type 3 SMA	• Type 2 or non-ambulatory Type 3 SMA • Receiving treatment with approved SMN upregulator	• Type 2 SMA • Initiated treatment with approved SMN upregulator before age 5
Primary Objectives	• Safety • Mean change from baseline in RHS	• Safety • Mean change from baseline in HFMSE	• Safety • Mean change from baseline in HFMSE

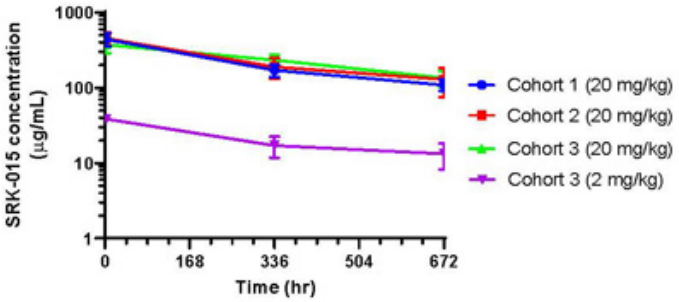
HFMSE – Hammersmith Functional Motor Scale Expanded; RHS – Revised Hammersmith Scale

Preliminary PK Data Shows SRK-015 Exposure in Patients with SMA Consistent with that in Healthy Volunteers

SRK-015 Displayed Well-Behaved, Linear PK Profile

- Minimal variability across TOPAZ cohorts
- Preliminary PK observations consistent with Phase 1 trial results in healthy volunteers
- Dose proportional increase in serum drug exposure between low (2 mg/kg) and high (20 mg/kg) doses

Preliminary TOPAZ Phase 2 Pharmacokinetic (PK) Data



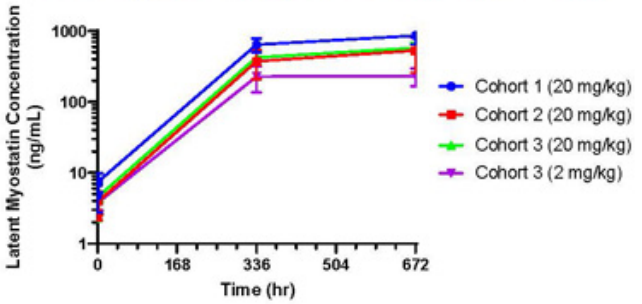
Preliminary PK/PD results include data from 29 patients (12 in Cohort 1, 8 in Cohort 2, and 9 in Cohort 3)

Preliminary Biomarker Data Provide First Demonstration of Target Engagement in Patients with SMA

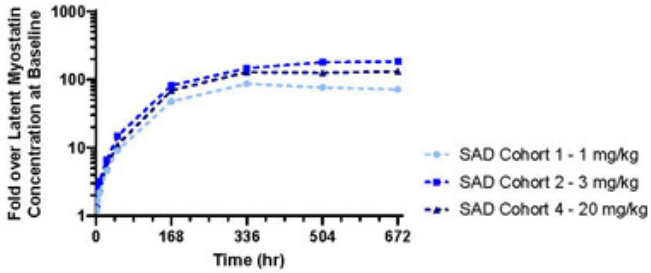
Robust Target Engagement Observed in TOPAZ

- ~100-fold increases in serum latent myostatin levels following a single 20 mg/kg dose in all cohorts
- Increases in latent myostatin were dose-dependent
- Confirms presence of latent myostatin in patients with SMA
- Fold increase from baseline in first 4 weeks after SRK-015 dose were comparable between SMA patients in TOPAZ and healthy volunteers in Phase 1

Latent Myostatin Change over Baseline in TOPAZ Trial

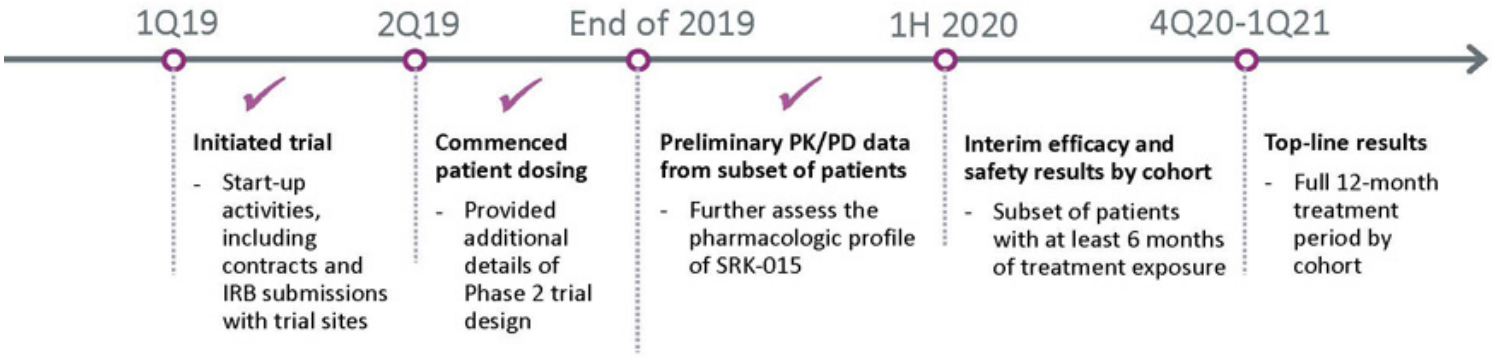


Latent Myostatin Change over Baseline in Phase 1 HV Trial



Preliminary PK/PD results include data from 29 patients (12 in Cohort 1, 8 in Cohort 2, and 9 in Cohort 3)

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



Initiated trial
- Start-up activities, including contracts and IRB submissions with trial sites

Commenced patient dosing
- Provided additional details of Phase 2 trial design

Preliminary PK/PD data from subset of patients
- Further assess the pharmacologic profile of SRK-015

Interim efficacy and safety results by cohort
- Subset of patients with at least 6 months of treatment exposure

Top-line results
- Full 12-month treatment period by cohort

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

TGFβ1: Significant Opportunities in Oncology/Immuno-oncology

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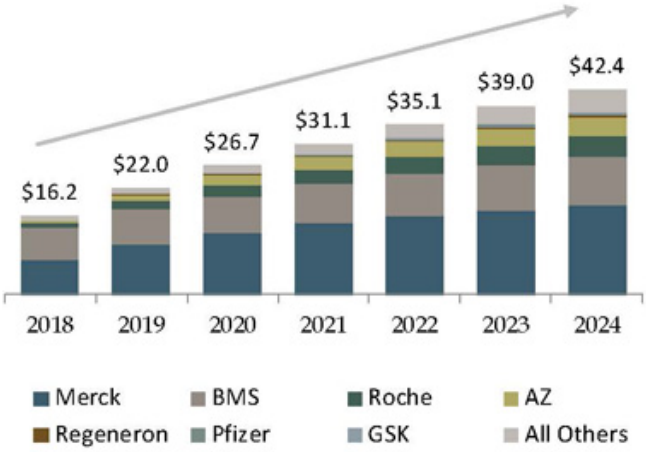
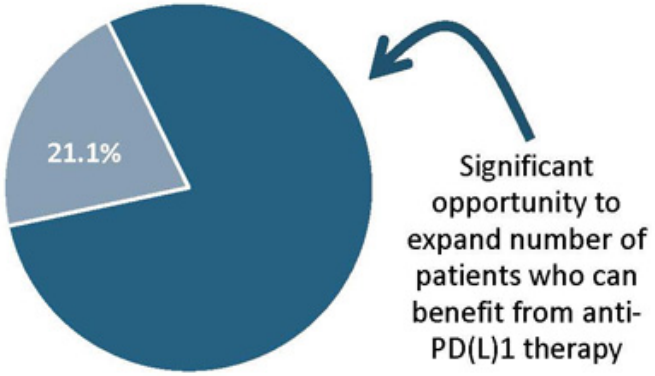
Anti-PD(L)1 Becoming Backbone Therapy Across Oncology; Significant Unmet Need Remains

Checkpoint Market Expected to Nearly Triple Over Next Few Years…



Source: Company information, Wall Street research, Evaluate Pharma

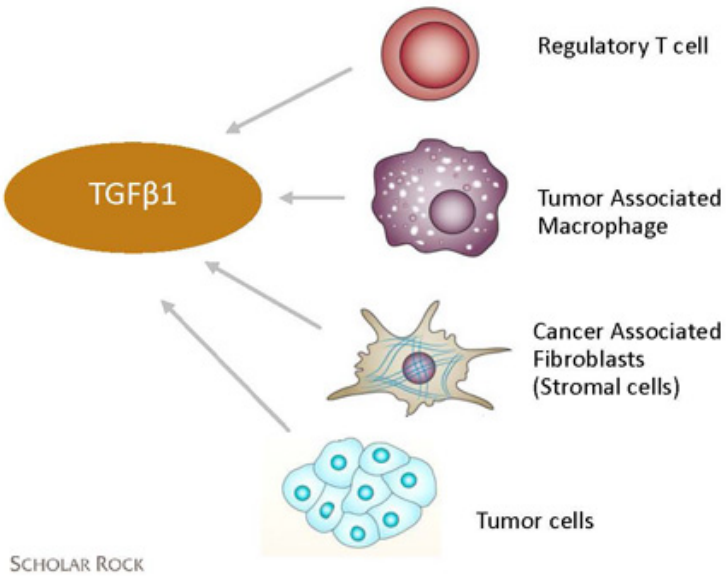
Yet only 21% of patients show complete or partial response to anti-PD(L)1 therapy



Source: Carretero-Gonzalez et al. (2018) Oncotarget 9:8706-8715
Meta-analysis of twelve randomized trials with control arm or adequate safety profile (includes nivolumab, pembrolizumab, and atezolizumab)

Inhibition of TGFβ1: Multipronged Approach for Immuno-Oncology

TGFβ1 is a key driver of immune system evasion by cancer cells



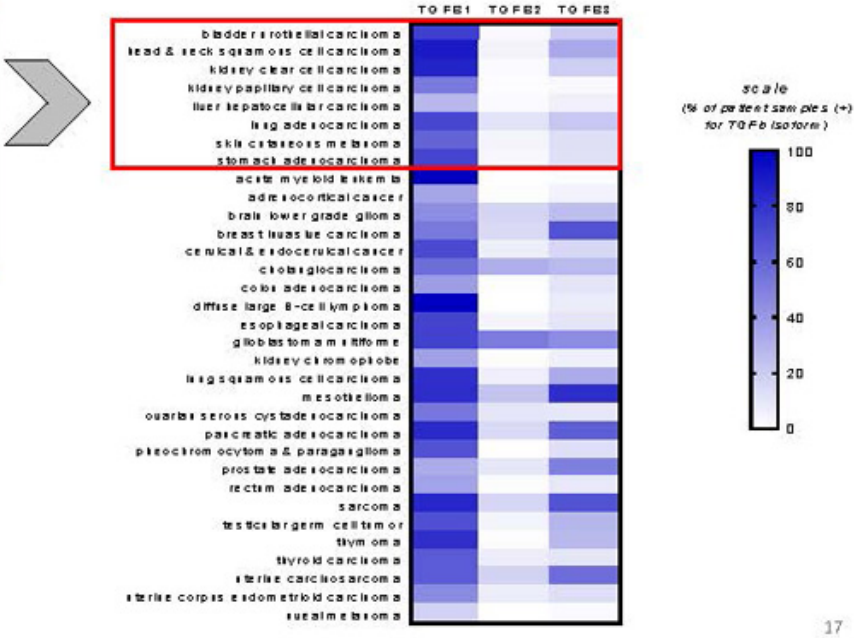
- Pathway analysis in patient tumors points to TGFβ1 as major determinant of primary resistance to anti-PD(L)1
- TGFβ1 creates 'immune excluded' tumor microenvironment

Selective inhibition of TGFβ1 activation has the potential to significantly increase anti-PD(L)1 response rates

TGFβ1 is the Predominant Isoform in Most Human Tumors

The Cancer Genome Atlas RNAseq analysis: >10,000 samples spanning 33 tumor types

- TGFβ1 prevalent in human cancers for which checkpoint therapies are approved
- Expression data for most tumor types suggest that TGFβ signaling mainly driven by TGFβ1



SRK-181 has Potential to Meaningfully Expand Number of Patients Who Could Benefit from Checkpoint Inhibitors

Differentiated approach with SRK-181

- *Highly selective inhibitor of the activation of latent TGFβ1*
- *Minimal or no binding to latent TGFβ2 and TGFβ3 isoforms*
- *Fully human mAb*

1Q20: Initiate Phase 1 trial in patients with locally advanced or metastatic solid tumors

Preclinical models show potential of SRK-181 in overcoming primary resistance to checkpoints

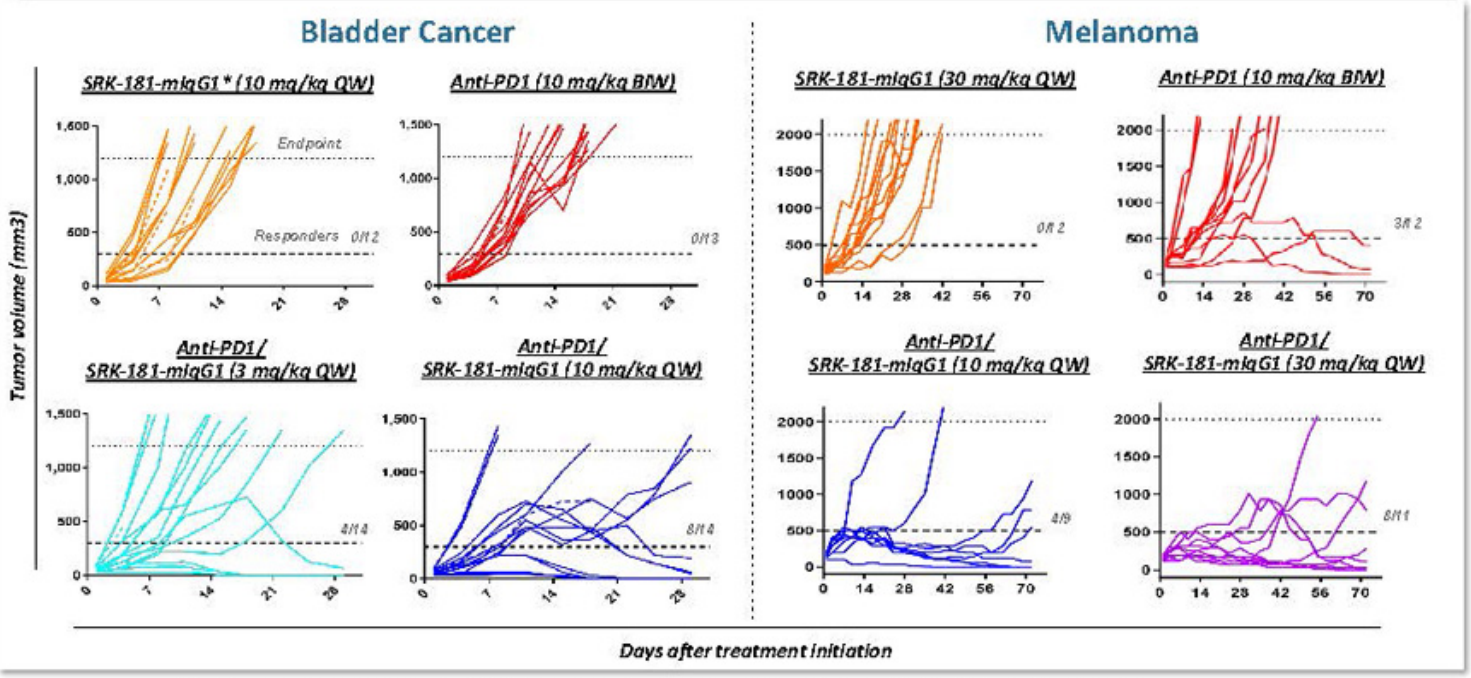
Treatment with SRK-181-mIgG1*:

- Rendered resistant TGFβ1- and TGFβ1/TGFβ3 co-expressing tumor models sensitive to anti-PD1
- Led to tumor regression and survival benefit when combined with anti-PD1
- Enabled infiltration and expansion of CD8+ T cells when combined with anti-PD1
- Avoided cardio toxicity associated with traditional less selective approaches

Syngeneic mouse tumor models specifically selected to reflect human tumor biology

*SRK-181-mIgG1 is the murine version of SRK-181

TGFβ1 Blockade with SRK-181-mIgG1 Rendered Preclinical Tumor Models Susceptible to Anti-PD1 Therapy



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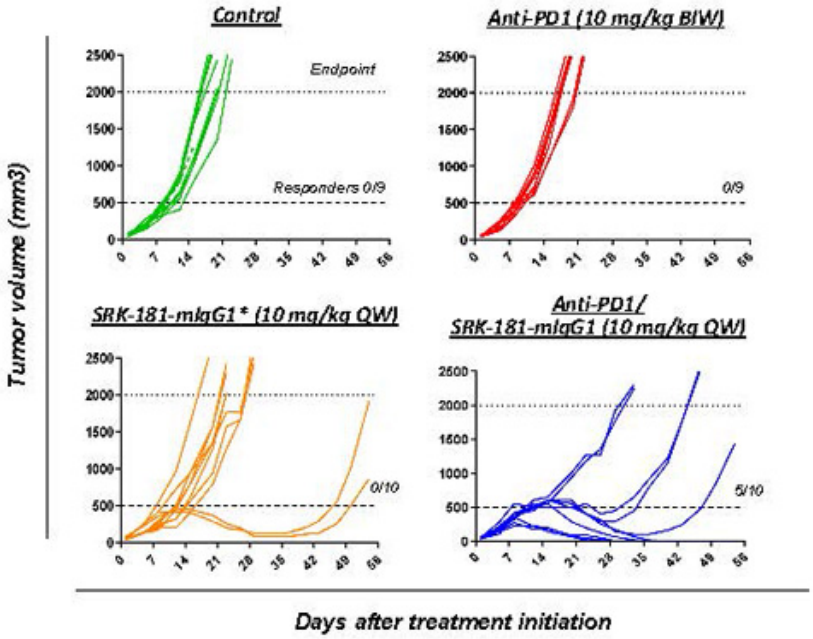
Preclinical data shown above as presented at American Association of Cancer Research (AACR) Annual Meeting (April 2019)
*SRK-181-mIgG1 is the murine version of SRK-181; Responder defined as tumor size <25% endpoint volume at study end

Inhibiting TGFβ1 Alone Was Sufficient to Sensitize Preclinical TGFβ1/3-Expressing Breast Cancer Model

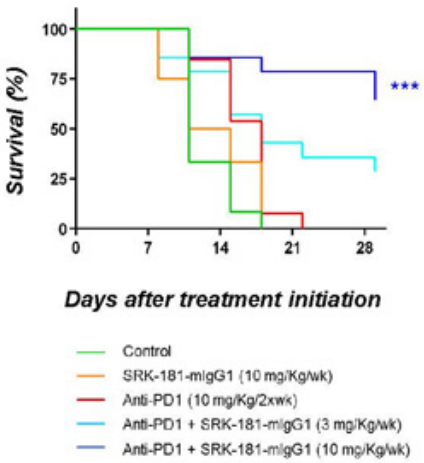


- EMT6 model expresses both TGFβ1 and TGFβ3
- Model is poorly responsive to PD1 blockade as a monotherapy
- Combination of SRK-181-mIgG1 and anti-PD1 resulted in tumor regression or tumor control

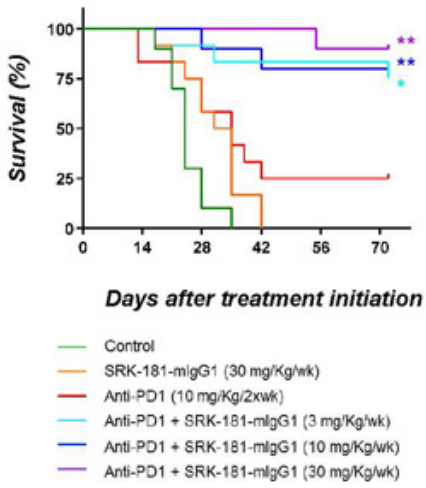
Preclinical data shown above as presented at American Association of Cancer Research (AACR) Annual Meeting (April 2019)
*SRK-181-mIgG1 is the murine version of SRK-181

SRK-181-mIgG1† Combined with Anti-PD1 Therapy Led to Significant Survival Benefit in Preclinical Tumor Models

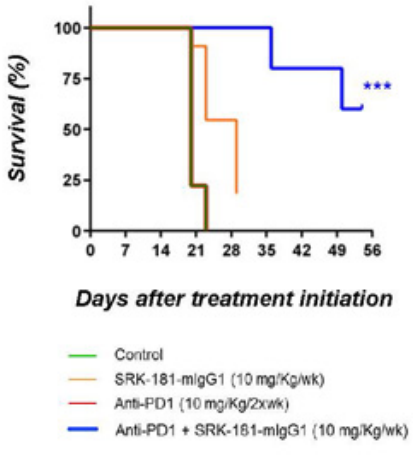
MBT-2 Bladder Cancer Model



Cloudman S91 Melanoma Model



EMT6 Breast Cancer Model



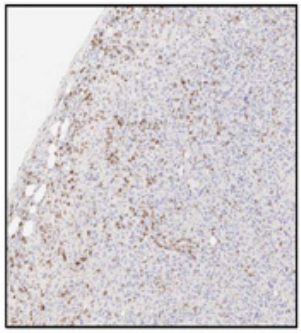
* P<0.05 Log-rank (Mantel-Cox test) vs. anti-PD1 **P<0.01 *** P<0.001

SCHOLAR ROCK

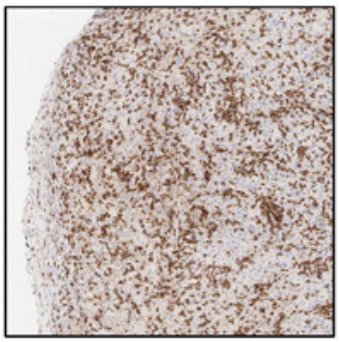
Preclinical data shown above as presented at American Association of Cancer Research (AACR) Annual Meeting (April 2019)
†SRK-181-mIgG1 is the murine version of SRK-181

SRK-181-mIgG1 Combination Therapy Enabled Infiltration and Expansion of CD8+ T cells in Preclinical Bladder Cancer Model

Anti-PD1



Anti-PD1/SRK-181-mIgG1 (10 mg/kg) led to increase in CD8+ cells



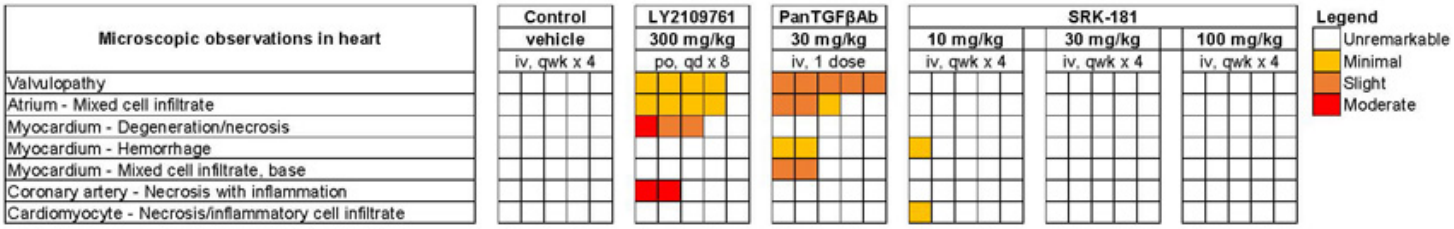
Combination treatment with SRK-181-mIgG1/anti-PD1 led to:

- Significant increase in effector T cells ($p<0.05$)
  - Expansion of CD8+ population to an average of 34% of the tumor's immune cells from a control average of 3.5%
- Significant decrease in intratumoral immunosuppressive myeloid cells ($p<0.05$)
  - Reduction in TAM/MDSC population to 14% of the tumor's immune cells from a control average of 47%
  - Reduction in MDSC population to 1.4% from 11% of CD45+ cells in the IgG control group

Preclinical data shown above as presented at American Association of Cancer Research (AACR) Annual Meeting (April 2019)
*SRK-181-mIgG1 is the murine version of SRK-181; Anti-PD1 dosed at 10 mg/kg twice weekly and SRK-181-mIgG1 dosed at 10 mg/kg weekly

TGFβ1 Isoform Specificity of SRK-181 Improved Preclinical Toxicity Profile

Repeat dose pilot toxicology study in adult female Sprague Dawley rats



- Animals dosed with pan-TGFβ inhibitors, LY2109761 (inhibitor of ALK5, common TGFβ receptor kinase) or pan-TGFβ antibody, exhibited expected cardiac findings based on published data
- Exposure as assessed by SRK-181 serum concentration reached 2,300 µg/ml following 4 weekly doses of 100 mg/kg
- No SRK-181 related adverse effects were noted up to 100 mg/kg per week
- No cardiotoxicities (valvulopathy) were noted with SRK-181
- No observed adverse effect level (NOAEL) for SRK-181 was the highest dose evaluated (100 mg/kg QW)

RGMc Program: Targeting the BMP6 Signaling Pathway

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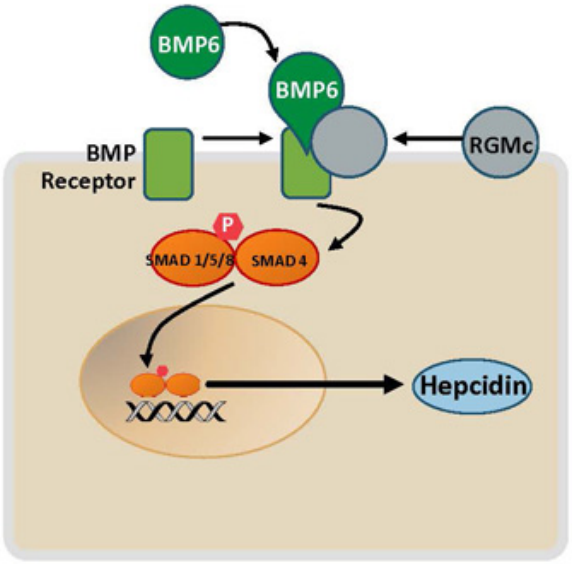
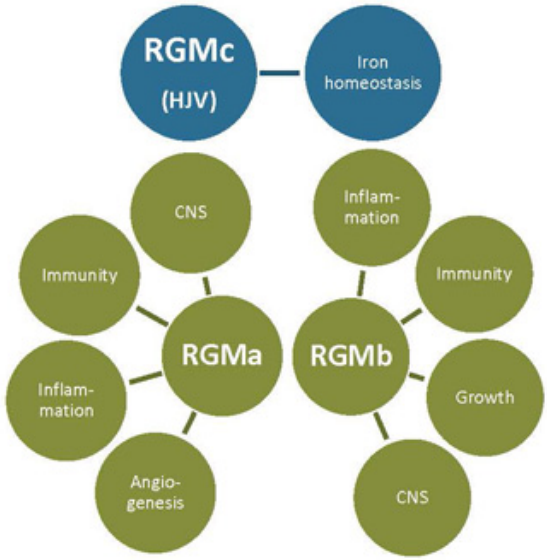
Anti-RGMc Therapy: Rational Solution that Directly Addresses the Underlying Pathobiology of Iron-Restricted Anemias

Genetically validated pathway of iron regulation in humans



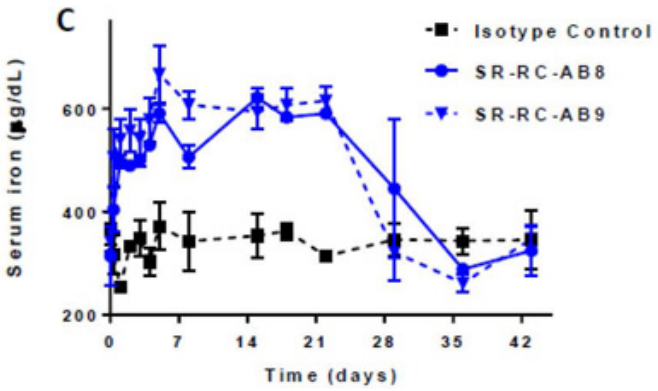
Adapted from Crielaard et al, Nature Reviews , 2017

RGMa and RGMb play many different physiological roles

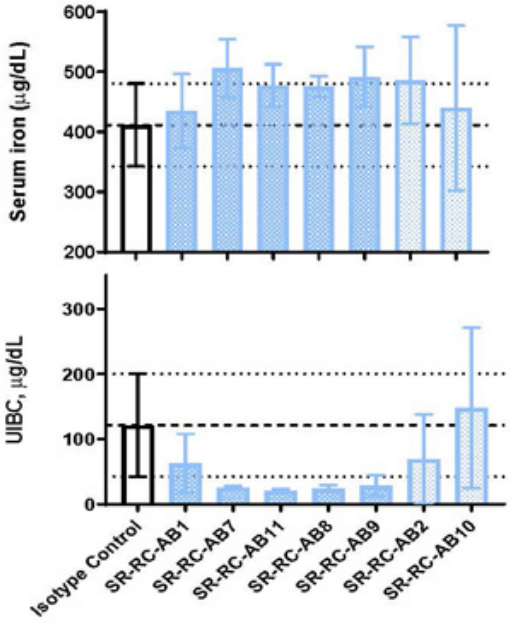


Scholar Rock's RGMc-Selective Antibodies Show Potent Increase in Serum Iron In Vivo

Antibodies showed up to 3 weeks prolonged increase in serum iron in SD rats (single 20 mpk dose)



Antibodies resulted in increases in serum iron and decreases in UIBC



Upcoming Key R&D Milestones

Spinal Muscular Atrophy (SMA)

- ✓ Initiate SRK-015 Phase 2 proof-of-concept SMA trial by the end of 1Q19
- ✓ Commence patient dosing in SRK-015 Phase 2 SMA proof-of-concept trial in 2Q19
- ✓ Present final Phase 1 results at Cure SMA Annual Conference being held June 28-July 1, 2019
- • Announce SRK-015 Phase 2 trial read-outs:
  - ✓ Preliminary PK/PD analysis by end of 2019
  - ○ Interim efficacy and safety results in a subset of patients at 6 months in 1H20
  - ○ Top-line results of 12-month treatment period 4Q20-1Q21

Oncology

- ○ Initiate SRK-181 Phase 1 dose escalation and POC trial in patients with solid tumors in 1Q20
- ○ Continue to advance active discovery programs for context-dependent inhibition of TGFβ1

Fibrosis

- ○ Continue to conduct fibrosis discovery and preclinical studies in partnership with Gilead

Anemias

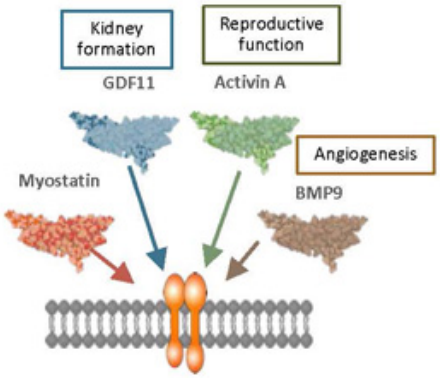
- ○ Nominate product candidate in RGMc program in 2020

Appendix

Traditional Approaches Can Raise Significant Safety Concerns

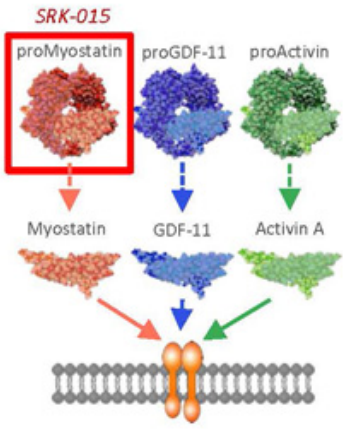
Traditional Approaches Lack Selectivity

- Most inhibitors of active myostatin also inhibit GDF11 and may inhibit other growth factors as well
- Antibodies to ActRIIb and ligand trap approaches inhibit signaling of multiple ligands

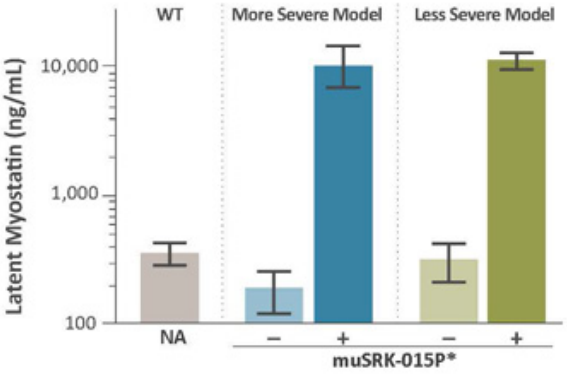


Scholar Rock Approach

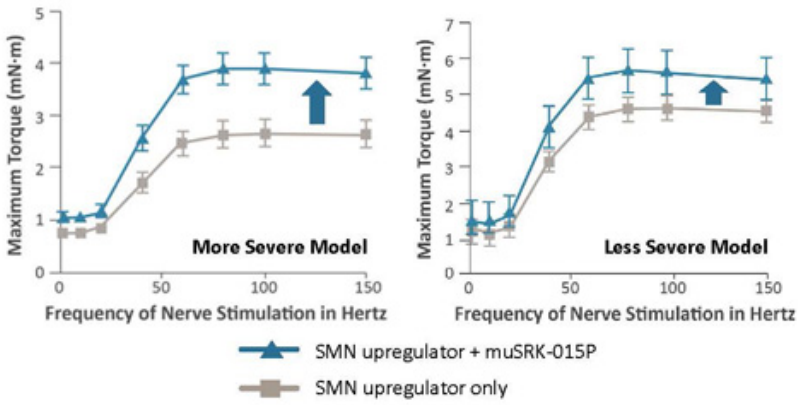
Exquisite Selectivity By Targeting Precursor Form of Myostatin



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%

*muSRK-015P is the parental clone of SRK-015 on a mouse IgG1 framework
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 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

SRK-015 Preclinical and Phase 1 Data Support Evaluation in SMA

Preclinical and translational data support myostatin as a drug target in SMA

- Confirmed presence of target in disease setting
- Achieved multi-fold increase in serum latent myostatin levels indicating target engagement
- Treatment of SMNΔ7 mouse model led to improved muscle mass and strength

Phase 1 data in healthy volunteers showed robust target engagement and no apparent safety signals

- No dose-limiting toxicities identified up to highest evaluated dose of 30 mg/kg
- Serum half-life of 23-33 days supports planned evaluation of once every 4-week (Q4W) dosing in Phase 2
- Single dose of SRK-015 led to marked increases in serum concentrations of latent myostatin; no meaningful change observed with placebo
- **Target saturation**: peak latent myostatin levels plateaued starting with a single dose at 3 mg/kg
- **Durability of saturation**: plateau was sustained up to Day 140 after multiple doses at 20 mg/kg

Phase 1 results provide first proof-of-mechanism in humans of Scholar Rock's therapeutic approach of targeting the latent form of growth factors

SRK-015 Well Tolerated in Phase 1 Healthy Volunteer Trial

- **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 treatment-related adverse events (AEs)
  - No treatment-related SAE
  - No hypersensitivity reactions
- **Anti-drug antibody tests were negative in all SRK-015 treated subjects**
- **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 35% (7/20) 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

*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

Phase 1 Pharmacokinetic (PK) Data Support Infrequent Dosing

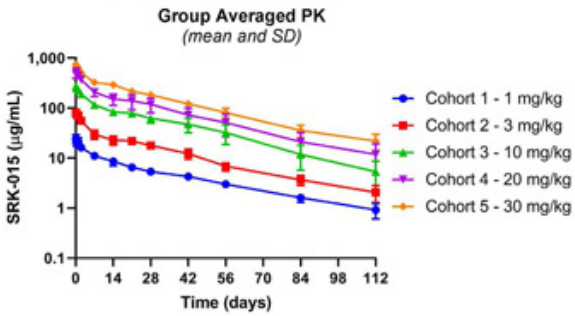
SRK-015 Displayed Well-Behaved, Linear PK Profile

- Minimal variability observed, consistent with that commonly observed with monoclonal antibodies
- Dose-proportional serum drug exposure

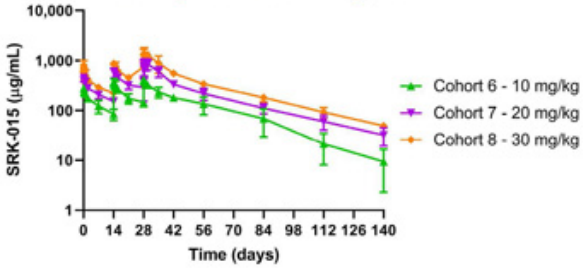
Half-Life Supports Infrequent Dosing

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

Single-Ascending Dose



Multiple-Ascending Dose



Phase 1 Pharmacodynamic (PD) Data Demonstrate Robust and Sustained Target Engagement

Robust Target Engagement Observed

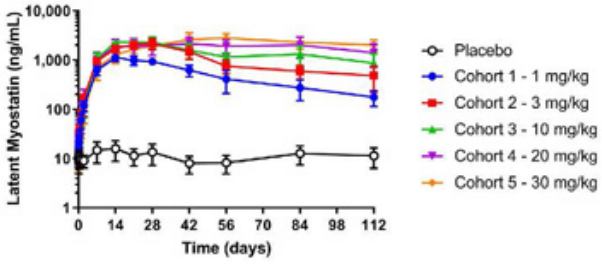
- Single dose of SRK-015 led to marked increases in serum concentrations of latent myostatin
- No meaningful change observed with placebo

Evidence Supports Durable Target Saturation

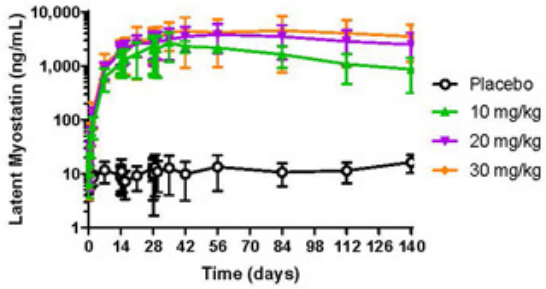
- Peak latent myostatin levels plateaued starting with a single dose at 3 mg/kg suggesting target saturation
  - Single dose at 1 mg/kg only attained approx. half of peak level
- Plateau was sustained demonstrating durability of effect:
  - Up to Day 84 after single dose at 20 mg/kg
  - Up to at least Day 140 after multiple doses at 20 and 30 mg/kg

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

Single-Ascending Dose



Multiple-Ascending Dose



TGFβ1 Plays Central Role in Multiple Diseases with Unmet Need

SCHOLAR ROCK

Oncology

- Immuno-Oncology
- Tumor-Directed Therapy
- Myeloproliferative Disorders

SCHOLAR ROCK

GILEAD

Fibrosis

Upfront cash and equity investment:
$80 million*

One-time preclinical milestone:
$25 million

Additional milestones across 3 programs:
Up to $1,425 million

High single- to low double-digit tiered royalties on net sales

Includes $30 million purchase of Scholar Rock common stock at price per share of $30.60