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Deep Insights
Impactful Medicines

January 2020

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Our Purpose

Relentlessly focused on seeing new possibilities in validated biologies and antibody technologies to allow us to move with speed and urgency to deliver transformative medicines to patients with devastating diseases

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Revolutionary Approach

- First company to successfully target growth factor precursors
- Pursue high value targets proven challenging for traditional non-selective approaches
- Focus on biologically validated targets using proven modality (mAbs)
- Leverage protein science and antibody expertise to develop high-impact medicines for patients suffering neuromuscular disorders, cancer, fibrosis and anemia
- Develop broad IP portfolio covering compositions and methods

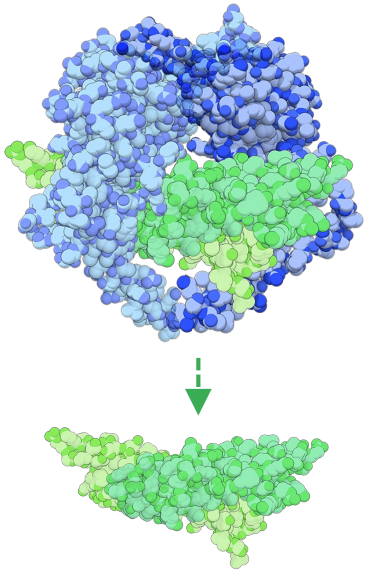
Differentiated Pipeline Portfolio

- Two lead product candidates, SRK-015 and SRK-181, in clinical development in 2020
- Multiple clinical read-outs offer near-term value inflection points
- Potential to expand pipeline with additional indications for each product candidate
- Strategic fibrosis collaboration with Gilead to develop potent and selective inhibitors of latent TGFβ activation
- Applying expertise across the TGFβ superfamily and beyond

Differentiated Approach to Highly Sought After Growth Factors Implicated in Devastating Diseases

Scholar Rock's Target

Growth Factor Precursor (Latent Form)



Targeting the activation of growth factor precursors to :

- Optimize potency and selectivity
- Localize effect

Traditional Target
"Mature" Growth Factor

Spinal Muscular Atrophy

SRK-015: Inhibitor of latent myostatin activation

Phase 2 Trial Ongoing TOPAZ

Immuno-Oncology

SRK-181: Inhibitor of latent TGFβ1 activation

Phase 1 Proof-of-Concept trial to initiate in 1Q20

Fibrosis

Inhibitors of latent TGFβ activation

Advancing collaboration with Gilead towards product candidate selection

Growing number of preclinical and clinical successes using Scholar Rock's differentiated approach

2019: Year of Execution and Progress

Advanced SRK-015 Towards Phase 2 Proof-of-Concept Data

- Initiated and completed enrollment in TOPAZ Phase 2 proof-of-concept trial
  - 3 cohorts of patients with Type 2 and Type 3 SMA
- Announced compelling preliminary PK/PD data from TOPAZ trial
- Presented positive data from Phase 1 healthy volunteer trial

Accelerated SRK-181 Towards Phase 1 Proof-of-Concept Trial

- Filed SRK-181 IND to FDA and plan to initiate a Phase 1 POC trial in patients with solid tumors in 1Q20
- Presented highly encouraging preclinical data in immuno-oncology with potent and selective inhibitor of latent TGFβ1 activation
- Completed pilot rat tox study demonstrating differentiated preclinical safety profile

Achieved First Milestone in Gilead Fibrosis Collaboration

- Advanced collaboration with successful demonstration of efficacy in preclinical in vivo proof-of-concept studies in fibrotic indications
- Earned $25M milestone; eligible to receive up to an additional $1,425M in potential payments from Gilead

2020/2021: Transformative Years with Multiple Clinical Read-Outs

Initiation of SRK-181 Phase 1 POC trial

- Two part trial with dose escalation (Part A) and dose expansion cohorts (Part B)
- Patients with locally advanced or metastatic solid tumors
- Exhibit primary resistance to anti-PD-(L)1 antibodies
- Evaluate multiple cancers, such as urothelial carcinoma, melanoma, and NSCLC, in combination with an approved anti-PD-(L)1 therapy

Interim efficacy and safety results from SRK-015 TOPAZ trial

- All 3 fully enrolled cohorts
- 6 months of treatment exposure

Initial clinical data from SRK-181 Phase 1 POC trial

- Part A clinical data

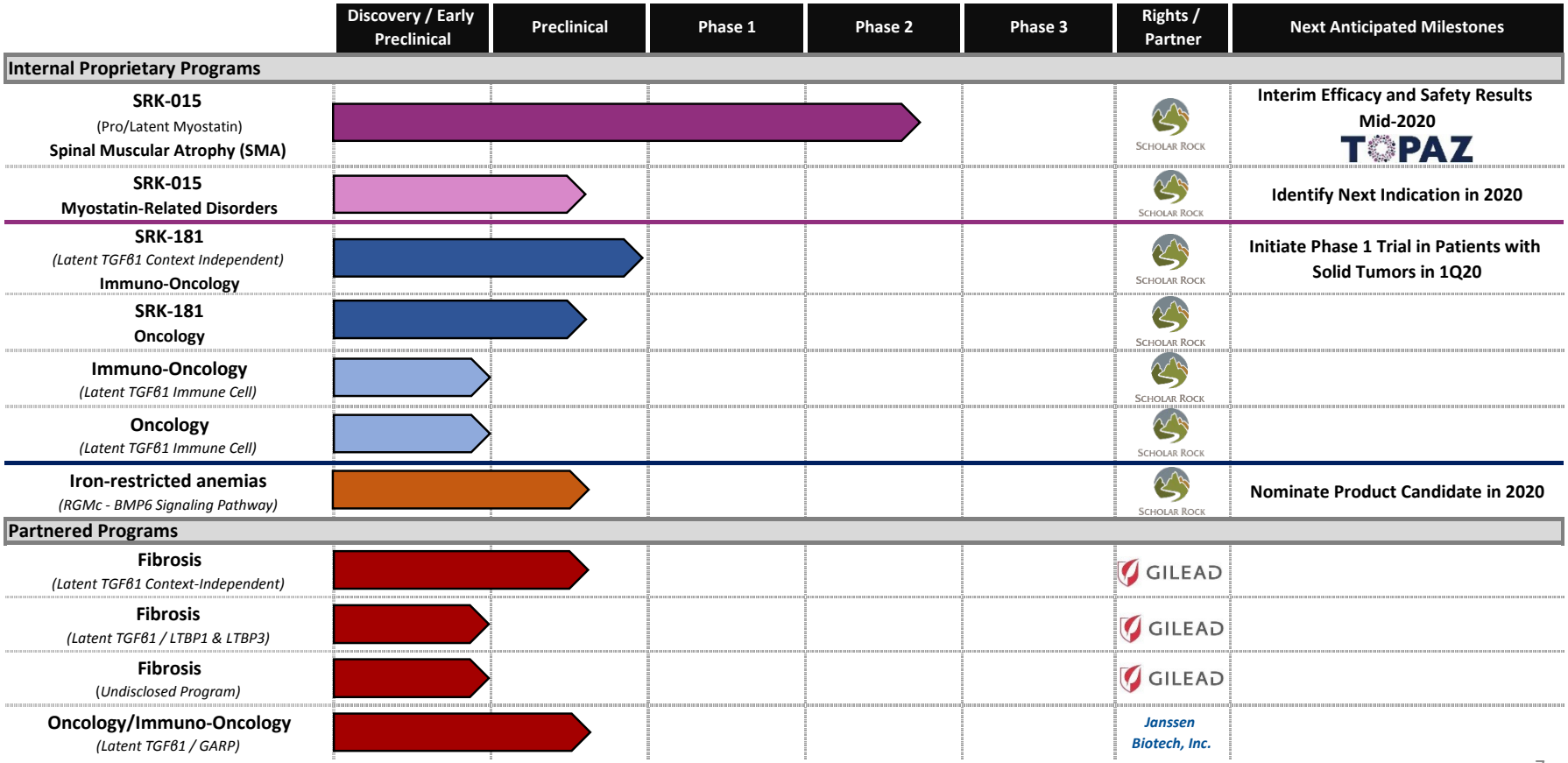
Top-line 12-month results from SRK-015 TOPAZ trial

- Full 12-month treatment period
- Patients eligible to continue treatment for an additional 12 months

Clinical response and safety data from SRK-181 Phase 1 POC trial

- Part B clinical data

Building Differentiated Pipeline; Pairing Revolutionary Approach with Proven Modality



SRK-181: Potential Transformative Backbone for a New Era of Cancer Immunotherapy

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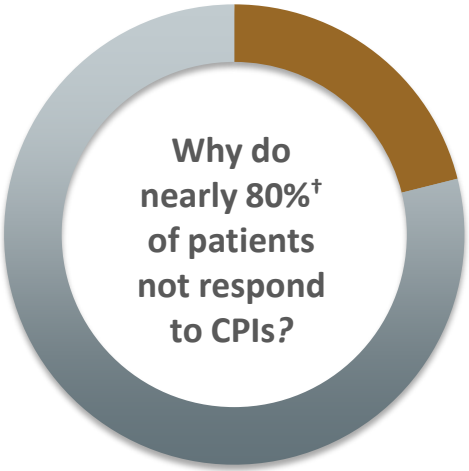
Human Tumor Analyses Reveal TGFβ as Key Determinant of Primary Resistance to Checkpoint Inhibitor (CPI) Therapies



TGFβ1 creates "immune-excluded" tumor microenvironment

Nature (on-line), Feb. 14, 2018

TGFβ attenuates tumour response to PD-L1 blockade by contributing to exclusion of T cells

Sanjeev Mariathasan[1]*, Shannon J. Turley[1]*, Dorothee Nickles[1]*, Alessandra Castiglioni[1], Kobe Yuen[1], Yulei Wang[1], Edward E. Kadel III[1], Hartmut Koeppen[1], Jillian L. Astarita[1], Rafael Cubas[1], Suchit Jhunjhunwala[1], Romain Banchereau[1], Yagai Yang[1], Yinghui Guan[1], Cecile Chalouni[1], James Ziai[1], Yasin Şenbabaoğlu[1], Stephen Santoro[1], Daniel Sheinson[1], Jeffrey Hung[1], Jennifer M. Giltnane[1], Andrew A. Pierce[1], Kathryn Mesh[1], Steve Lianoglou[1], Johannes Riegler[1], Richard A. D. Carano[1], Pontus Eriksson[2], Mattias Höglund[2], Loan Somarriba[3], Daniel L. Halligan[3], Michiel S. van der Heijden[4], Yohann Loriot[5], Jonathan E. Rosenberg[6], Lawrence Fong[7], Ira Mellman[1], Daniel S. Chen[1], Marjorie Green[1], Christina Derleth[1], Gregg D. Fine[1], Priti S. Hegde[1], Richard Bourgon[1] & Thomas Powles[8]

Article

Cell

Genomic and Transcriptomic Features of Response to Anti-PD-1 Therapy in Metastatic Melanoma

Authors

Willy Hugo, Jesse M. Zaretsky, Lu Sun, ...,
Douglas B. Johnson, Antoni Ribas, Roger S. Lo

Volume 165, Issue 1, 24 March 2016, Pages 35-44

Clinically-derived rationale points to significant opportunity to increase checkpoint therapy responses

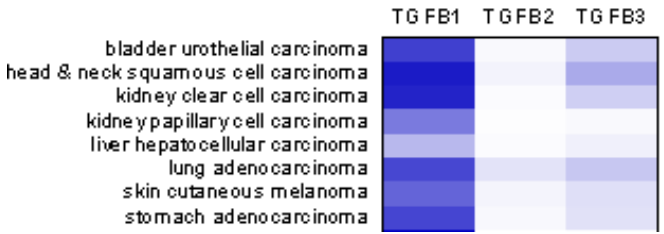
† Source: Carretero-Gonzalez et al. (2018) Oncotarget 9:8706-8715
Meta-analysis of twelve randomized trials with control arm or adequate safety profile (includes nivolumab, pembrolizumab, and atezolizumab)

Implicating TGFβ1 as the Resistance Culprit and Selecting Preclinical Models with Clinically Relevant Features

TGFβ1 is most likely driver of TGFβ pathway signaling in human cancers

- TGFβ1 most prevalent isoform in most human cancers
- TGFβ1 expression correlates with TGFβ pathway activity in tumors

Cancer Genome Atlas RNAseq analysis of >10,000 samples spanning 33 tumor types†



Matching syngeneic mouse tumor models to human tumor biology††

Phenotype of Resistant Human Tumors in αPD-(L)1 Therapies

- Immune exclusion
- Minimal or no response to anti-PD-(L)1
- High TGFβ1 over TGFβ2/3 expression
- Evidence for TGFβ signaling

Selection Criteria for Mouse Tumor Models

- Immune exclusion
- Minimal or no response to anti-PD-(L)1
- High TGFβ1 over TGFβ2/3 expression
- Evidence for TGFβ signaling

Aim to pick the right target and improve likelihood of translatability from preclinical models to patients

†Source: National Cancer Institute - Cancer Genome Atlas Program
††Source: Mariathasan, Turley, et.al TGFβ attenuates tumour response to PD-L1 blockade by contributing to exclusion of T cells, Nature (online), Feb 2018

Differentiated Approach with SRK-181

- *Fully human mAb*
- *Potent and selective inhibitor of latent TGFβ1 activation*
- *Minimal or no binding to latent TGFβ2 and TGFβ3 isoforms*
- *Designed for exquisite selectivity for TGFβ1 to avoid the cardiac tox often seen with traditional, less-selective approaches*

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Strong preclinical data shows potential of SRK-181 in overcoming primary resistance to checkpoints

- ~80% of patients with metastatic solid tumors do not respond to CPIs[†]
- Strong human translational data and preclinical models implicate TGFβ as key culprit in primary resistance to CPIs[††]
- Exquisite selectivity of SRK-181 offers potential to reduce toxicity and avoid dose-limiting challenges
- Rationally designed preclinical studies* demonstrate potential of SRK-181 in overcoming primary resistance

SRK-181 IND submitted; plan to initiate Phase 1 POC trial in 1Q20

- Opportunities for data read-outs in 2H20 and 2021

Preclinical efficacy and safety results point toward a clinically feasible path forward

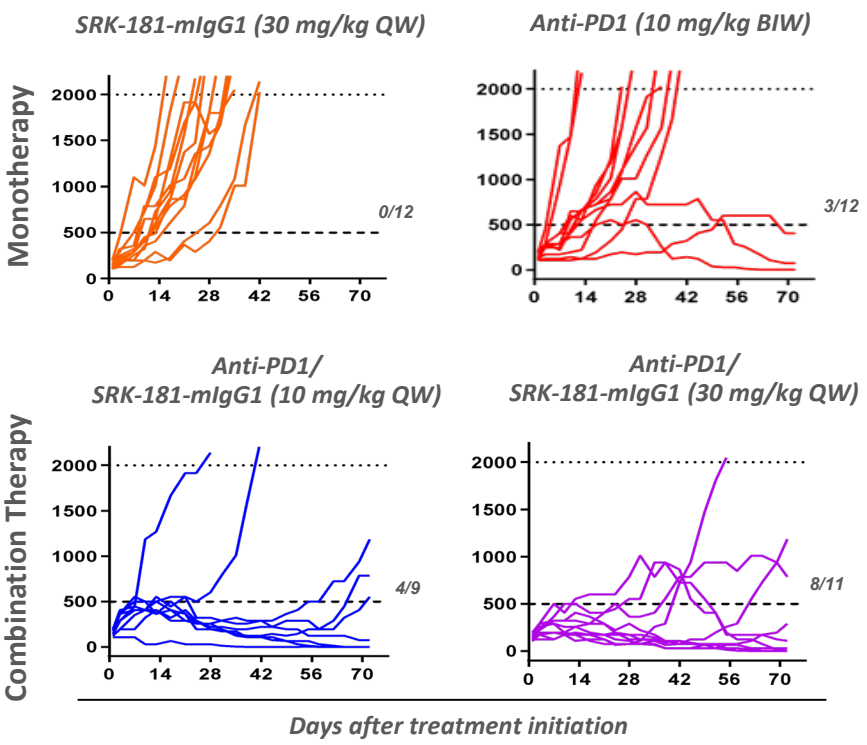
Refer to posters presented at SITC (Nov 2018) and AACR (April 2019) available at www.scholarrock.com

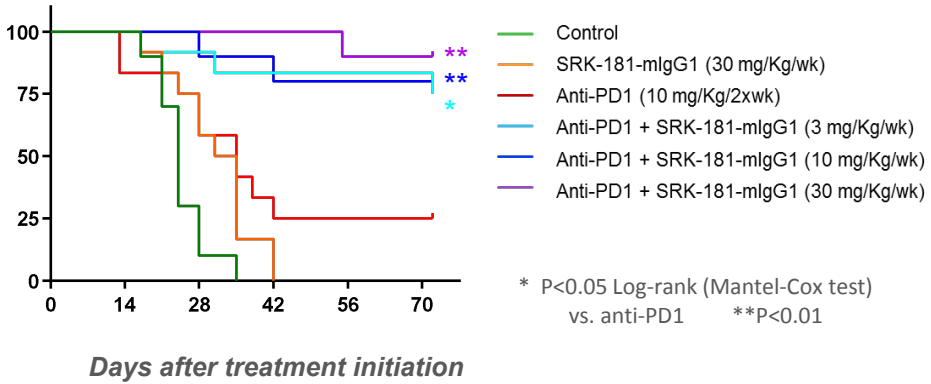
[†]Source: Carretero-Gonzalez et al. (2018) Oncotarget 9:8706-8715

[††]Source: Mariathasan, Turley, et.al *TGFβ attenuates tumour response to PD-L1 blockade by contributing to exclusion of T cells, Nature (online),* Feb 2018

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

Cloudman S91 melanoma model: Combination treatment led to tumor regression and survival benefit



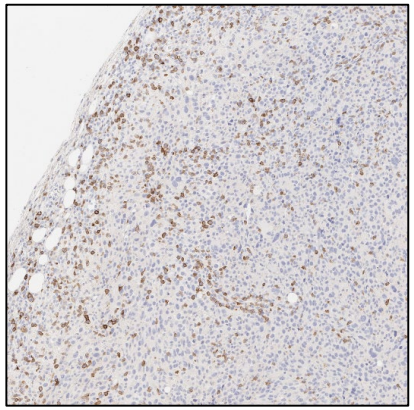


* P<0.05 Log-rank (Mantel-Cox test) vs. anti-PD1 **P<0.01

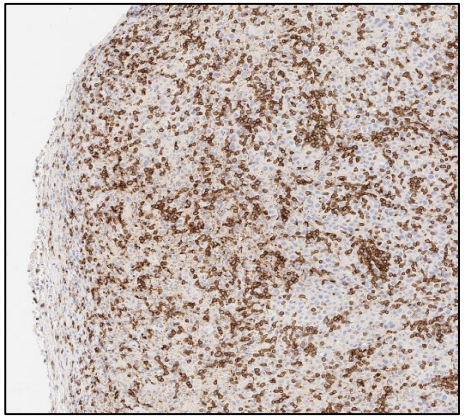
Similar results demonstrated in bladder model and breast cancer model (TGFβ1/3 co-expressing)

SRK-181-mIgG1 Combination Therapy Enabled Infiltration and Expansion of CD8+ T cells

Anti-PD1



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



Turning "cold" tumors "hot," and reduction in suppressive myeloid cells are consistent with significant anti-tumor responses

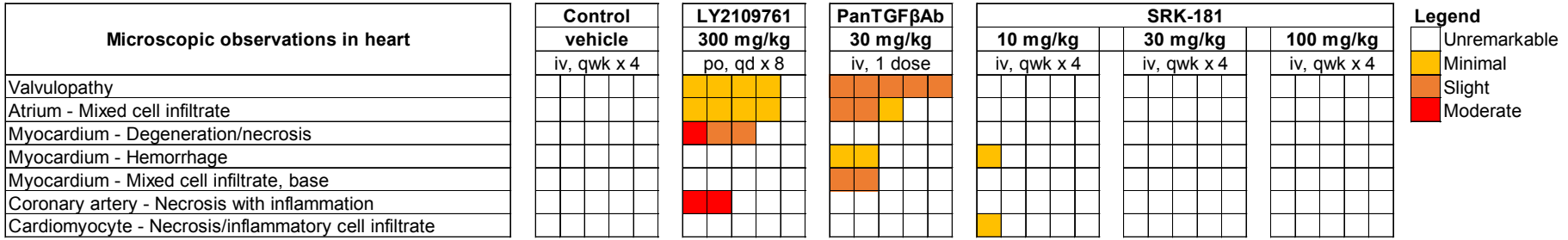
In preclinical bladder cancer model, combination treatment with SRK-181-mIgG1/anti-PD1 led to:

- 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)
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



- Cardiac findings were exhibited in animals dosed with an pan-TGFβ antibody or LY2109761 (inhibitor of ALK5, common TGFβ receptor kinase) as expected based on published data†
- SRK-181 exposure 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)

Selectivity of SRK-181 offers potential to overcome toxicity and dose-limiting challenges of non-selective TGFβ pathway approaches

Preclinical data shown above as presented at American Association of Cancer Research (AACR) Annual Meeting (April 2019)
†Source: Anderton, et al. *Induction of Heart Valve Lesions by Small-Molecule ALK5 Inhibitors, Toxicology Pathology,* 39: 916-924, 2011, *Stauber et al. Nonclinical Safety Evaluation of a Transforming Growth Factor β Receptor…J Clin Pract 2014: 4:3*

Phase 1 Trial to Evaluate SRK-181's Ability to Overcome Primary Resistance to CPIs

Phase 1 Proof-of Concept Trial

SRK-181: potent and selective inhibitor of TGFβ1 activation

- Evaluate as a cancer immunotherapy in combination with anti-PD-(L)1 antibodies
- Patients with locally advanced or metastatic solid tumors
- Exhibit primary resistance to anti-PD-(L)1 antibodies
- Focus on cancers for which checkpoint inhibitors are approved, such as urothelial carcinoma, melanoma, and non-small cell lung

Two-part clinical trial:

1) **Dose escalation** – single agent and in combination with an approved anti-PD-(L)1
2) **Dose expansion** – multiple tumor-specific cohorts evaluating SRK-181 with an approved anti-PD-(L)1

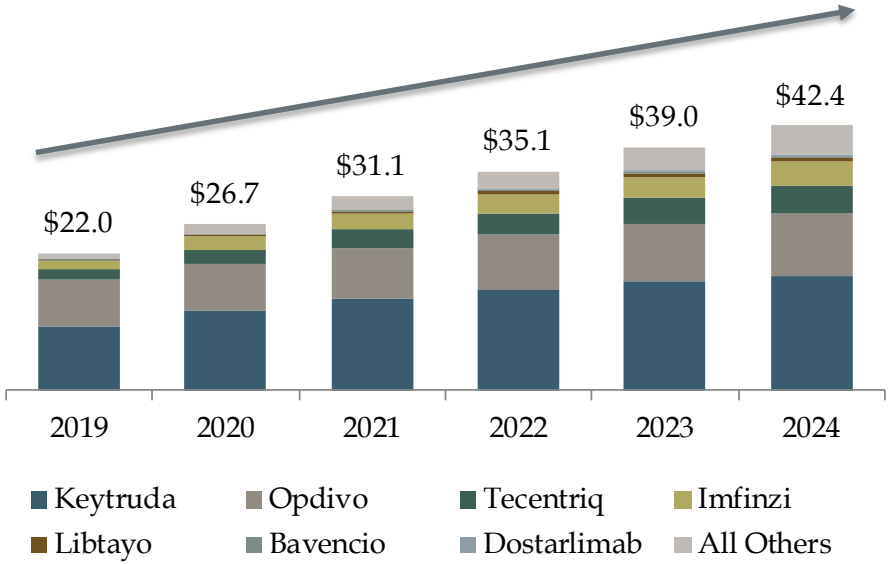
Initiate Phase 1 trial in 1Q20

Initial clinical data in 2H20

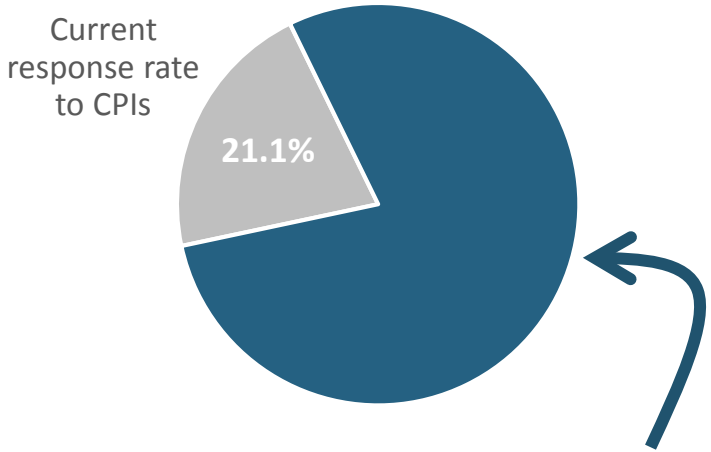
Clinical response and safety data in 2021

SRK-181 Has Potential to Increase Response to CPIs Through Clinically Relevant Resistance Pathway

Market for checkpoint inhibitor therapies expected to double over the next few years†…



…Yet Medical Need Not Addressed by Current Era of Immunotherapy††:



SRK-181 has potential to substantially increase the addressable patient population for all checkpoint therapies

†Source: Company information, Wall Street research, Evaluate Pharma
††Source: Carretero-Gonzalez et al. (2018) Oncotarget 9:8706-8715

SRK-015: Potential First Muscle-Directed Therapy for Spinal Muscular Atrophy

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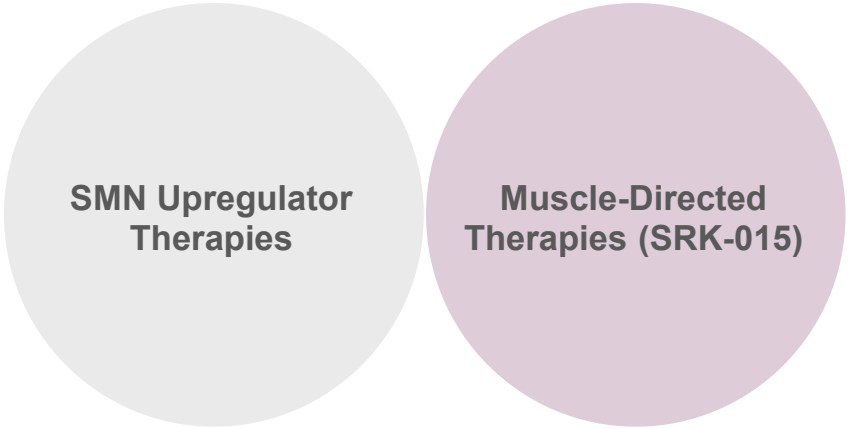
Significant Unmet Need Remains Despite Current Therapies

Muscle-Directed Therapies Needed to Complement Disease-Stabilizing Benefits of SMN Upregulators*

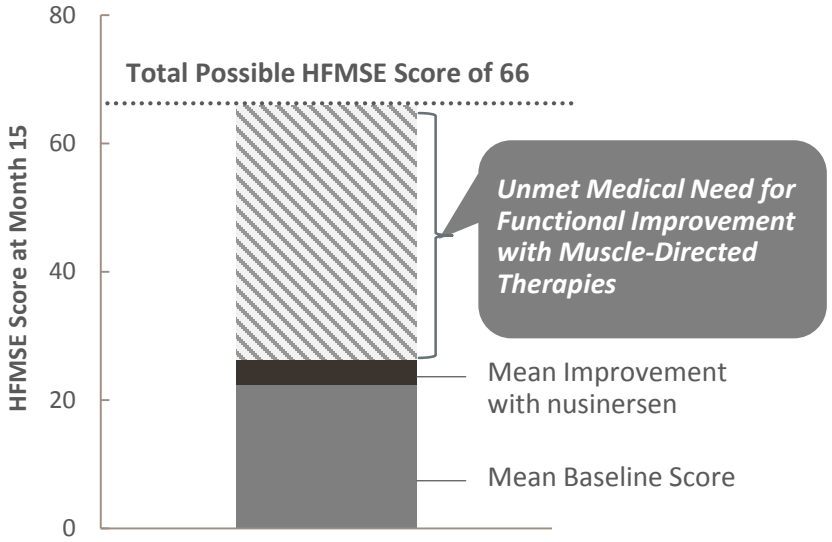


Address SMN deficiency to prevent further motor neuron deterioration

Act directly on muscle with aim to improve motor function

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

SMN = survival motor neuron
**also referred to as SMN correctors*

Source: Mercuri, E. et.al. Nusinersen versus Sham Control in Later-Onset Spinal Muscular Atrophy, N Engl J Med 2018; 378:625-635

SRK-015: Selective Inhibitor of Latent Myostatin Activation

- *Fully human mAb*
- *Half-life of 23-33 days*
- *Orphan Drug Designation for SMA granted by FDA and EC*
- *Strong patent portfolio:*
  - *US Patent 9,758,576 covers mAbs that inhibit the activation of myostatin precursor (expiry in 2034)*
  - *US Patent 10,287,345 covers treatment methods for various myostatin-related conditions (expiry in 2037)*

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Preclinical and clinical data provide strong rationale for developing in Spinal Muscular Atrophy (SMA)

- Need to improve motor function remains despite the availability of SMN upregulators for the treatment of SMA
- SMA disease features align well with attributes of myostatin biology
- Studies in SMNΔ7 mouse models demonstrated substantial increases in muscle strength
- Emerging pharmacologic profile for SRK-015 supports chronic therapy:
  - Evaluating Q4W dosing regimen
  - Phase 1/Phase 2 prelim. PD data show robust target engagement
  - No clinically significant safety signals observed as of data cutoff in preliminary Phase 2 PK/PD analysis

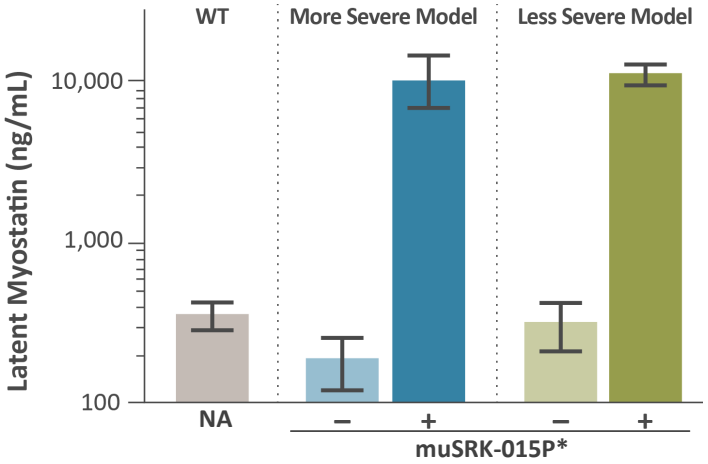
TOPAZ Phase 2 trial enrollment completed; Interim 6 month data mid-2020

- Top-line 12-month analysis anticipated 4Q20/1Q21

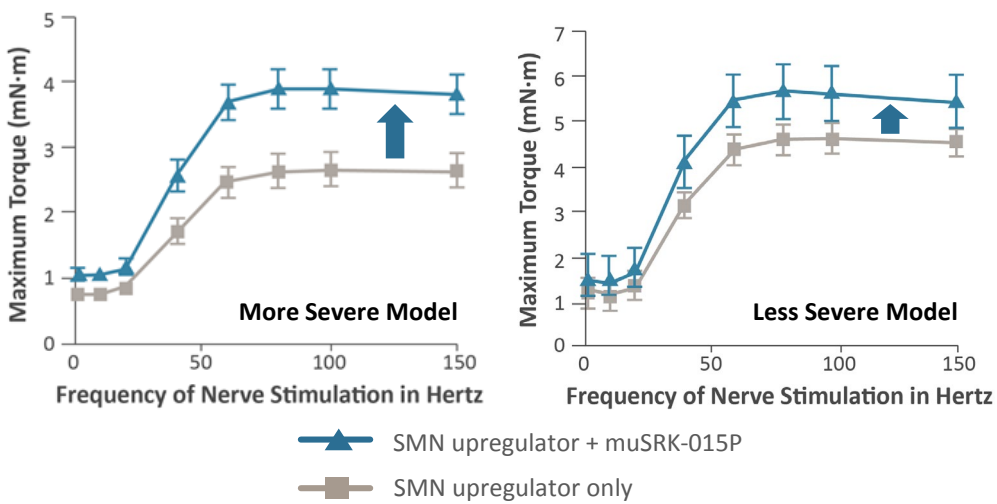
Therapeutic potential as muscle-directed therapy to complement any SMN upregulator

Source: Mercuri, E. et.al. Nusinersen versus Sham Control in Later-Onset Spinal Muscular Atrophy, N Engl J Med 2018; 378:625-635
Refer to Phase 1 data poster at World Muscle Society (Oct 2019) at www.scholarrock.com
Refer to press release announcing preliminary PK/PD data (Nov 19, 2019) at www.scholarrock.com

Treatment of SMNΔ7 Mouse Models Show Improvement in Muscle Strength



- 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 strength:

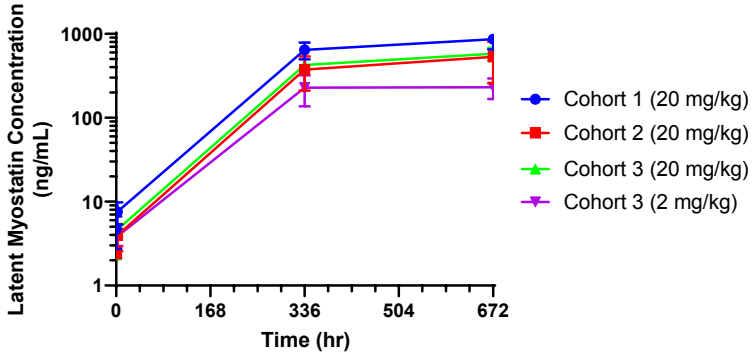
- Maximal torque of the plantar flexor muscle group increased:
  - More severe model: 44%-51%
  - Less severe model: 20%-30%

**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

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

Latent Myostatin Change over Baseline in SRK-015 TOPAZ Trial



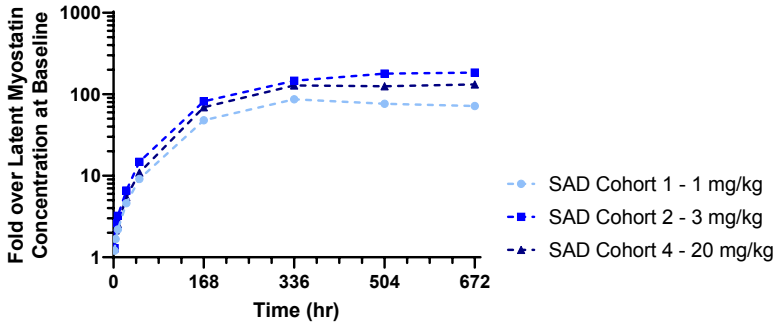
Robust Target Engagement Observed

- ~100-fold increases in serum latent myostatin levels following single 20 mg/kg dose in all cohorts
- Confirms presence of latent myostatin in patients with SMA

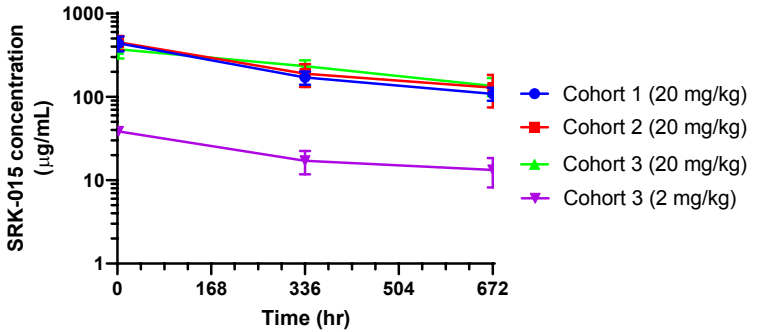
Well-Behaved, Linear PK Profile

- Minimal variability across TOPAZ cohorts
- Dose proportional increase in serum drug exposure between low (2 mg/kg) and high (20 mg/kg) doses

Latent Myostatin Change over Baseline in Phase 1 HV Trial



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)

Refer to press release announcing preliminary PK/PD data (Nov 19, 2019) at www.scholarrock.com

SRK-015 Phase 2 Trial Design

TOPAZ

Interim 6-Month Efficacy and Safety Results Mid-2020; Top-line 12-Month Data 4Q20/1Q21

	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
Patients	• Ambulatory Type 3 SMA • Receiving treatment with approved SMN upregulator or as monotherapy	• 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

3 point improvement in a patient on the HFMSE/RHS is considered clinically meaningful†

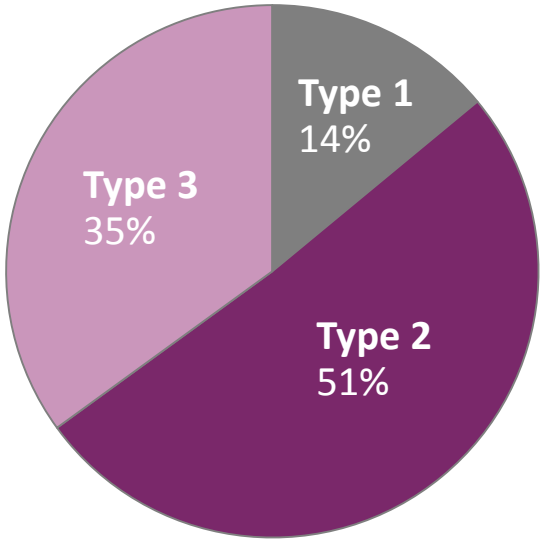
HFMSE – *Hammersmith Functional Motor Scale Expanded;* RHS – *Revised Hammersmith Scale*

†Source: Mercuri, E. et.al. Nusinersen versus Sham Control in Later-Onset Spinal Muscular Atrophy, N Engl J Med 2018; 378:625-635

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 1:

- Infant-onset; often fatal

Type 2 and non-ambulatory type 3:

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

Ambulatory type 3:

- Limited mobility and substantial morbidity

Type 4:

- Population not well-characterized

Focus of TOPAZ Trial

Potential to use SRK-015 in conjunction with SMN upregulators

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

…potential to expand more broadly in future trials

Lally et al, Orphanet Journal of Rare Diseases, 2017

Building Differentiated Pipeline Through Highly Productive Platform

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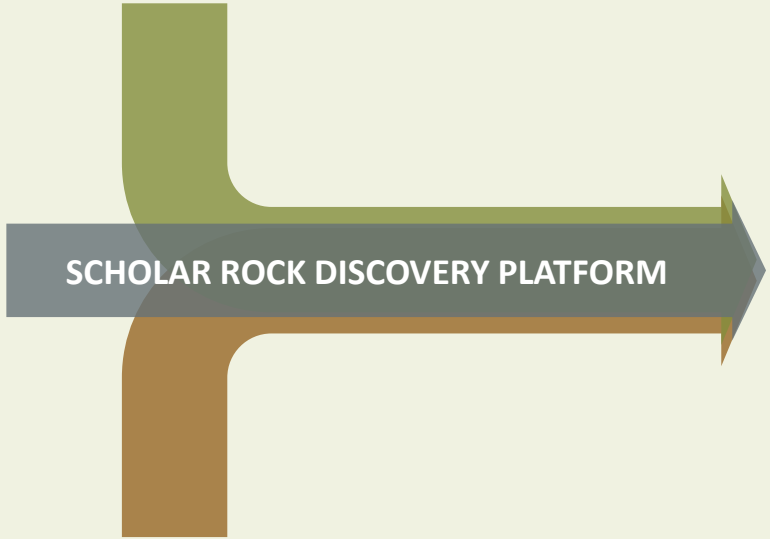
Proprietary Platform to Target Latent Growth Factor Activation

Design and Purification of Challenging Proteins
(e.g. latent growth factor complexes)

Proprietary Assays
that Recapitulate
Nature's Activation
In Vitro



Sophisticated Selection Strategies Utilizing
Recombinant Antibody Libraries

- Neuromuscular Disorders
- Oncology
- Fibrosis
- Anemia

Strategic collaboration focused on fibrosis

Gilead has exclusive options to license worldwide rights to product candidates from 3 TGFβ programs:

- *Inhibitors that target activation of latent TGFβ1*
- *Inhibitors that selectively target activation of latent TGFβ1 localized to extracellular matrix*
- *Undisclosed TGFβ discovery program*

SCHOLAR ROCK

Potent and Selective Inhibitors of Latent TGFβ Activation Can Offer Novel Approach to Fibrotic Diseases

- TGFβ-driven signaling has been broadly implicated as a central regulator of fibrosis†
- Scholar Rock's potent and highly selective TGFβ inhibitors act locally in the disease microenvironment
- Demonstrated preclinically that potent and selective inhibitors of TGFβ1 signaling prevent the activation of the growth factor in the fibrotic matrix
- Achieved first milestone and earned $25 million payment with demonstration of efficacy in in vivo proof-of-concept studies

Advance collaboration towards product candidate selection

†Kim KK, Sheppard D, Chapman HA (2018). TGF-β1 Signaling And Tissue Fibrosis. Cold Spring Harb Perspect Biol 10: a022293

Fibrosis Partnership with Gilead Advances with Achievement of First Milestone

Scholar Rock's highly specific inhibitors of latent TGFβ activation:

- Aim to improve efficacy and tolerability compared to traditional non-selective approaches
- Demonstrated efficacy in *in vivo* preclinical models

Upfront cash and equity investment:
$80 million*

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

One-time preclinical milestone:
$25 million
(achieved end of 2019)

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

SCHOLAR ROCK

- Antibody discovery and preclinical research thru product candidate nomination
- Distinct antibodies
- Retains exclusive WW rights for oncology and cancer immunotherapy

GILEAD

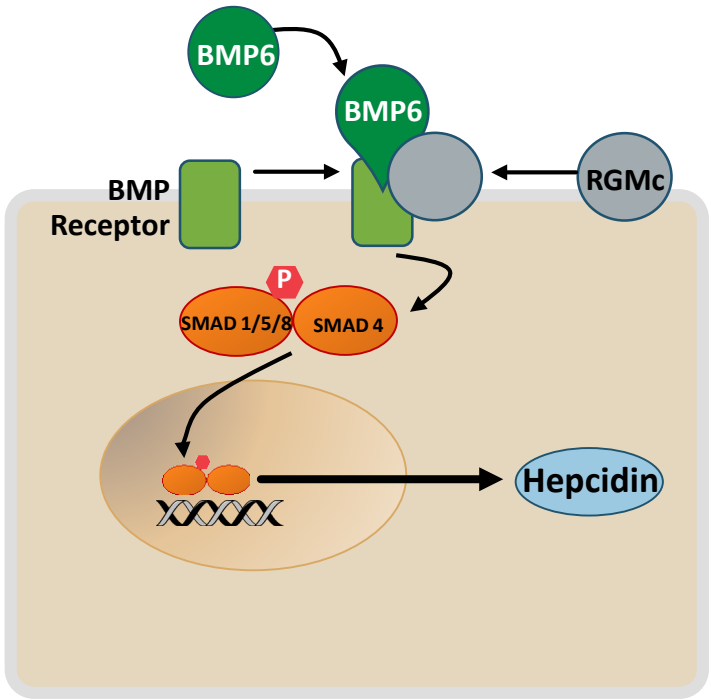
- Upon option exercise, responsible for preclinical and clinical development and commercialization

TGFβ-driven signaling broadly implicated as a central regulator of fibrosis

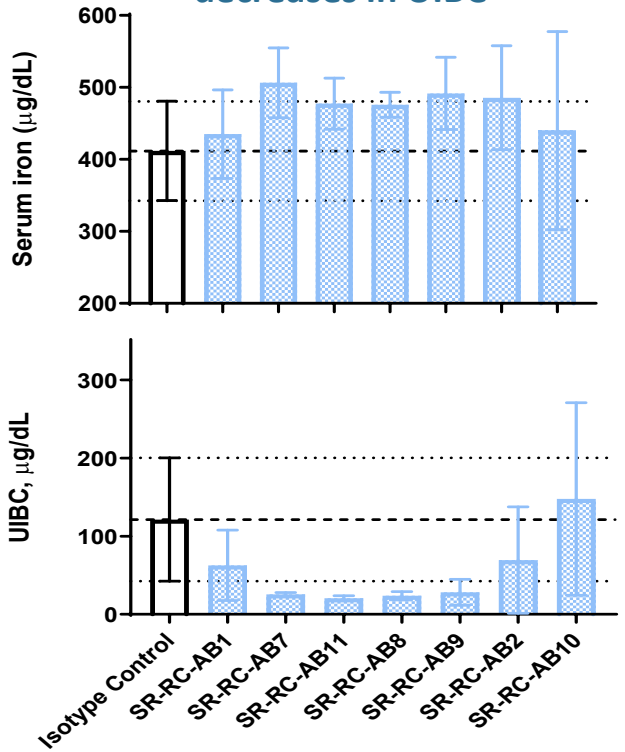
**Includes $30 million purchase of Scholar Rock common stock at price per share of $30.60*

Potential New Approach that Directly Addresses the Underlying Pathobiology of Iron-Restricted Anemias

Genetically validated pathway of iron regulation in humans



Antibodies resulted in increases in serum iron and decreases in UIBC*



Adapted from Crielaard et al, Nature Reviews , 2017

**Unsaturated iron binding capacity*
Refer to poster at International BMP Conference (Oct 2018) at www.scholarrock.com

Upcoming Key R&D Milestones

Spinal Muscular Atrophy (SMA)

- **Interim efficacy and safety results from SRK-015 TOPAZ Phase 2 trial in mid-2020**
  - All 3 fully enrolled cohorts with 6 months of treatment exposure
- **Top-line results (12 months) from SRK-015 TOPAZ trial in 4Q20-1Q21**
  - Patients are eligible to continue treatment for additional 12 months
- Identification of second indication for SRK-015 in 2020

Oncology

- **Initiate SRK-181 Phase 1 proof-of-concept trial in patients with solid tumors in 1Q20**
  - Patients that exhibit primary resistance to anti-PD-(L)1 antibodies
  - Evaluate multiple tumor types in combination with an approved anti-PD-(L)1 therapy
- Initial clinical data from SRK-181 Phase 1 POC trial in 2H20
- Clinical response and safety data from SRK-181 Phase 1 POC trial throughout 2021
- Continue to advance active discovery programs for context-dependent inhibition of TGFβ1

Fibrosis

- Continue to advance collaborative programs with Gilead towards product candidate selection

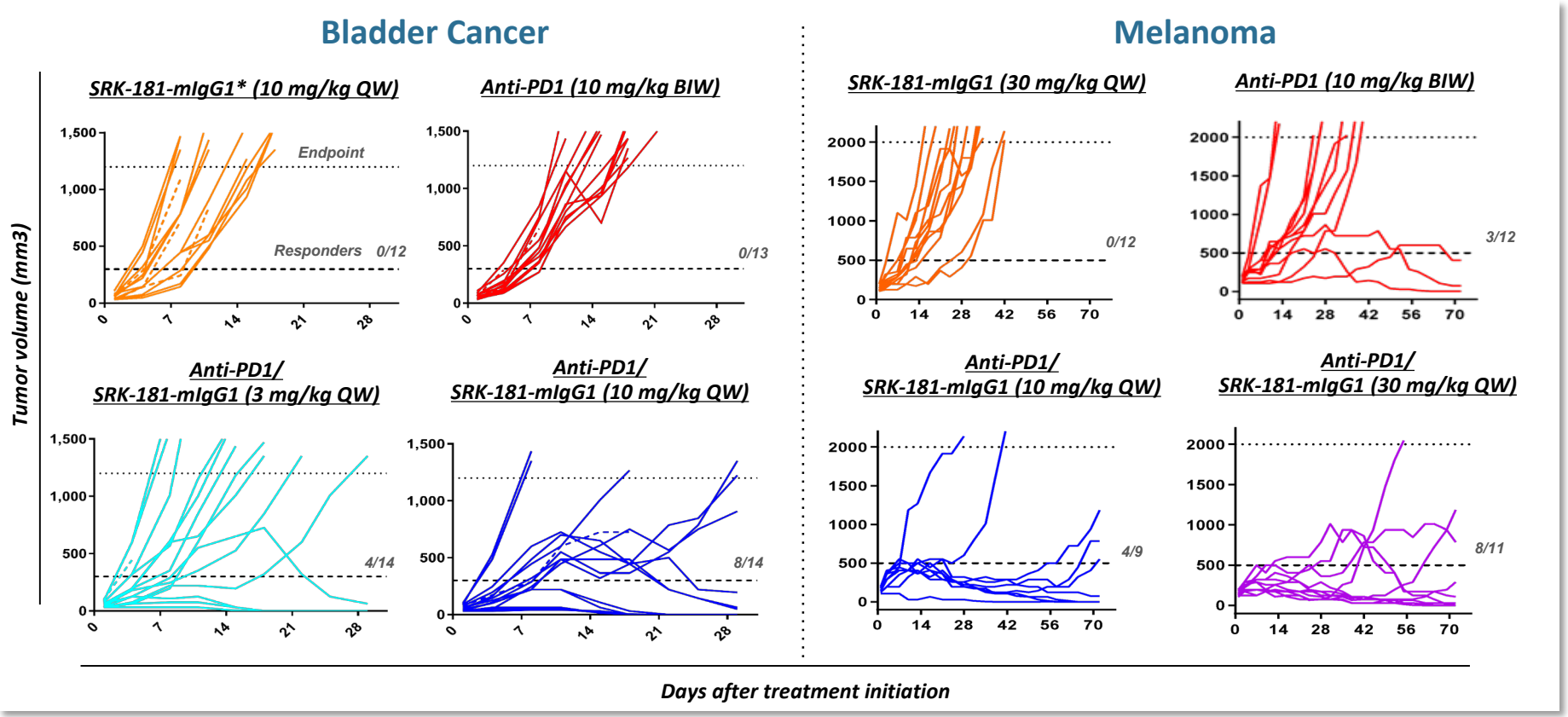
Anemias

- Nominate product candidate in RGMc program in 2020

Appendix

SCHOLAR ROCK

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

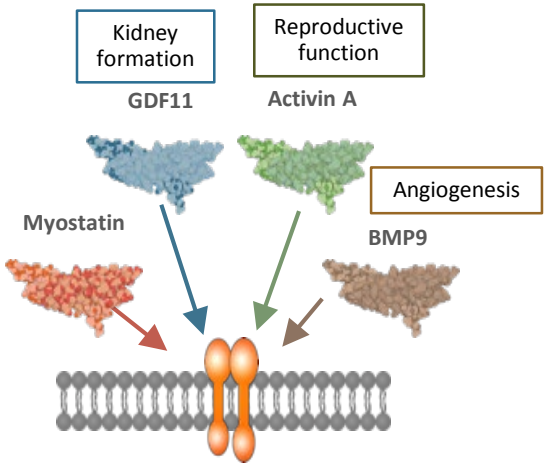


Preclinical data shown above as presented at American Association of Cancer Research (AACR) Annual Meeting (April 2019)
**SRK-181-mIgG1 is the murine version of SRK-181; Responder defined as tumor size <25% endpoint volume at study end*

Traditional Approaches Can Raise Significant Safety Concerns

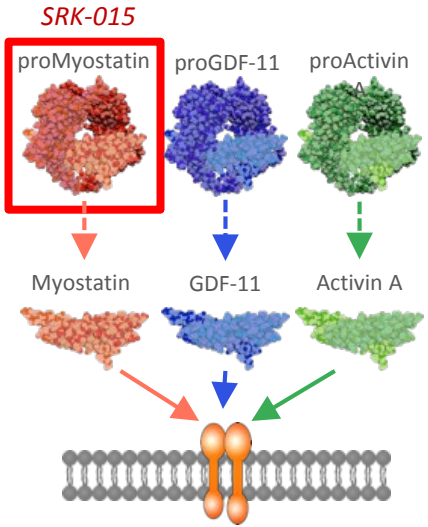
Traditional Approaches Lack Selectivity

- 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



To Date Demonstrates Proof-of-Mechanism of First Ever Approach to Targeting Latent Myostatin

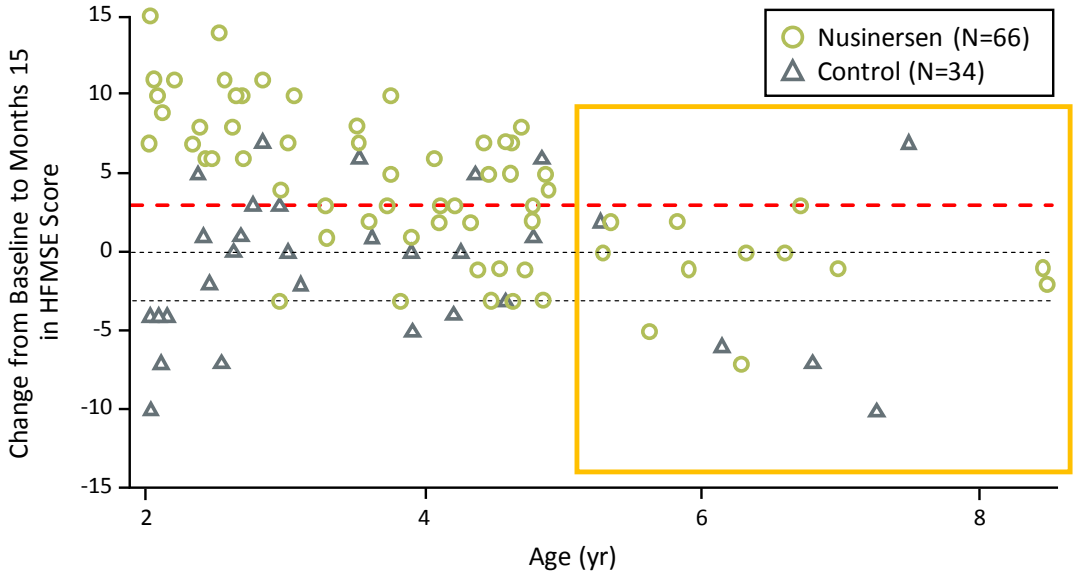
	SMNΔ7 Mouse Models	Phase 1 Healthy Volunteers	TOPAZ Phase 2 Trial Prelim PK/PD
Presence of latent myostatin target	Confirmed presence in diseased mice	Confirmed presence in humans	Confirmed presence in SMA patients
Target engagement	Multi-fold increases in serum latent myostatin levels	Robust and sustained target engagement	Dose-dependent increases of up to 100-fold; consistent with Ph1
Muscle strength or function	20%-51% increase in muscle strength (maximal torque)	NA	*Study ongoing*
Pharmacodynamic profile	NA	Serum half-life of 23-33 days	Well-behaved, linear PK profile; evaluating Q4W dosing
Safety / Adverse Events	None identified	No dose-limiting toxicities up to highest evaluated dose	No clinically significant safety signals observed

Preclinical and clinical evidence consistent thus far; TOPAZ interim efficacy/safety results mid-2020

Refer to preclinical and Phase 1 data poster at World Muscle Society (Oct 2019) at www.scholarrock.com
Refer to press release announcing preliminary PK/PD data (Nov 19, 2019) at www.scholarrock.com

Later-Onset SMA: High Unmet Need to Improve Motor Function

Nusinersen CHERISH Trial in Later-Onset SMA



In patients with later-onset SMA who were age ≥ 5 at screening…

- Primary benefit of nusinersen appeared to be stabilization of motor function (in HFMSE) rather than improvement from baseline
- Attainment of ≥ 3 point increase was very infrequent even with nusinersen treatment

3 point improvement in a patient is considered clinically meaningful

Source: Mercuri, E. et.al. Nusinersen versus Sham Control in Later-Onset Spinal Muscular Atrophy, N Engl J Med 2018; 378:625-635