Deep Insights, Impactful Medicines

July 2020

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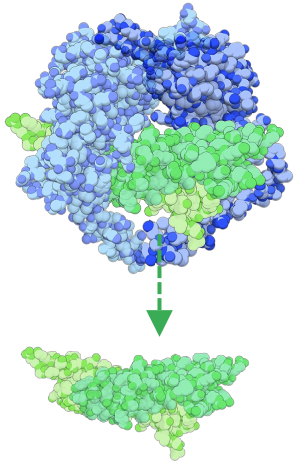
Bringing a Differentiated Approach to Highly Sought-After Growth Factors Implicated in Devastating Diseases

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

Immuno-Oncology

SRK-181: Inhibitor of latent TGFβ1 activation

DRAGON Phase 1 proof-of-concept trial ongoing

Spinal Muscular Atrophy

SRK-015: Inhibitor of latent myostatin activation

TOPAZ Phase 2 proof-of-concept trial ongoing

Fibrosis

Inhibitors of latent TGFβ activation

Advancing collaboration with Gilead towards product candidate selection

Growing preclinical and clinical progress using Scholar Rock's differentiated approach

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2020/2021: Transformative Years for Scholar Rock; Multiple Planned Clinical Read-Outs

1Q20

Initiated DRAGON 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

4Q20

Interim efficacy and safety results from TOPAZ SRK-015 trial

- Enrollment completed for all 3 cohorts in January*
- Analysis will be of patients who have progressed through at least 6 months of the treatment period

1H21

Update on dose escalation from DRAGON SRK-181 Phase 1 POC trial

- Part A: SRK-181 as a single agent and in combination with anti-PD-(L)1 therapy

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

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

2021

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

- Part A and Part B clinical data
- Multiple parallel cohorts to evaluate the anti-tumor activity of SRK-181 in combination with anti-PD-(L)1 therapy

*Demographics and baseline characteristics presented at the MDA Clinical and Scientific Conference (March 2020).https://scholarrock.com/platform/publications/.

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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 (online), 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 A, et al. *Oncotarget*. 2018;9:8706-8715. Meta-analysis of 12 randomized trials with control arm or adequate safety profile (includes nivolumab, pembrolizumab, and atezolizumab),

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Significant Industry Interest in Potential Role of TGFβ Inhibition in Immuno-Oncology

Feb. 5, 2019

"GSK and Merck KGaA, Darmstadt, Germany announce global alliance to jointly develop and commercialise M7824, a novel immunotherapy with potential in multiple difficult-to-treat cancers"

- *€300 million upfront*
- *Up to €3.7 billion total economics*

June 10, 2019

"Merck to Acquire Tilos Therapeutics: Merck Gains Portfolio of Investigational Antibodies Modulating TGFβ"

- *$773 million total potential deal value*

Two-year follow-up of bintrafusp alfa, a bifunctional fusion protein targeting TGF-β and PD-L1, for second-line (2L) treatment of non-small cell lung cancer (NSCLC)

B. C. Cho[1], T. M. Kim[2], D. Vicente[3], E. Felip[4], D. H. Lee[5], K. H. Lee[6], C.-C. Lin[7], M. J. Flor[8], M. Di Nicola[9], R. M. Alvarez[10], I. Dussault[11], C. Helwig[12], L. S. Ojalvo[11], J. L. Gulley[13], L. Paz-Ares[14]

[1]Yonsei Cancer Center, Yonsei University College of Medicine, Seoul, Korea; [2]Seoul National University Hospital, Seoul, Republic of Korea; [3]Hospital Universitario Virgen Macarena, Seville, Spain; [4]Hospital Universitari de la Vall d'Hebron, Barcelona, Spain; [5]Asan Medical Center, University of Ulsan College of Medicine Seoul, Seoul, Republic of Korea; [6]Chungbuk National University Hospital, Chungcheongbuk-Do, Republic of Korea; [7]National Taiwan University Hospital, Taipei, Taiwan; [8]Hospital Universitario Virgen del Rocío, Sevilla, Spain; [9]Fondazione IRCCS Istituto Nazionale Tumori, Milan, Italy; [10]Gregorio Marañon Hospital, Madrid, Spain; [11]EMD Serono Research & Development Institute, Inc., Billerica, MA, USA; a business of Merck KGaA, Darmstadt, Germany; [12]Merck KGaA, Darmstadt, Germany; [13]National Cancer Institute, National Institutes of Health, Bethesda, MD, USA; [14]Hospital Universitario 12 de Octubre, Madrid, Spain

Presented at ASCO 2020

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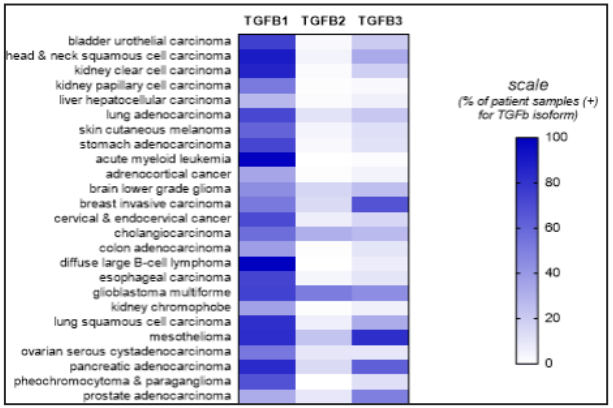
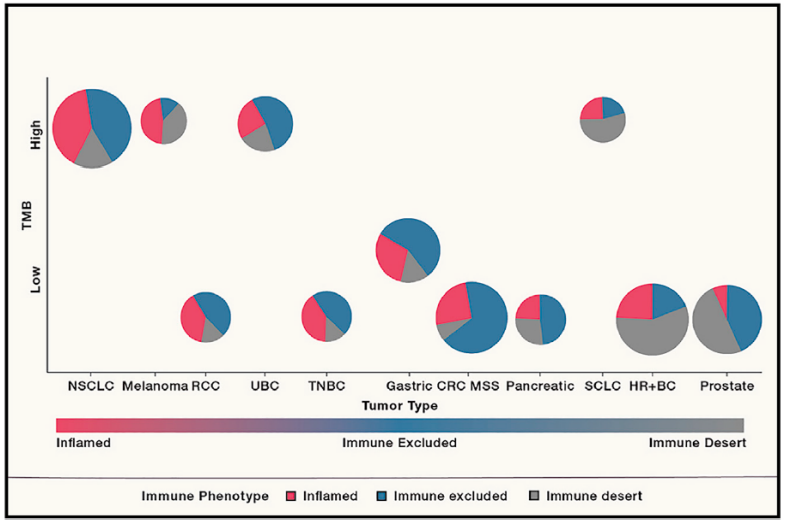
Broad Potential for TGFβ Blockade Across Many Solid Tumors

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



- TGFβ1 is the most likely driver of TGFβ pathway signaling in human cancers
- TGFβ1 expression correlates with TGFβ pathway activity in tumors

Substantial proportion of solid tumors exhibit immune exclusion†



*Source: National Cancer Institute - Cancer Genome Atlas Program.
†Priti H, et al. Top 10 challenges in cancer immunotherapy. *Immunity*. 2020 Jan 14:52(1):17-35. https://doi.org/10.1016/j.immuni.2019.12.011.

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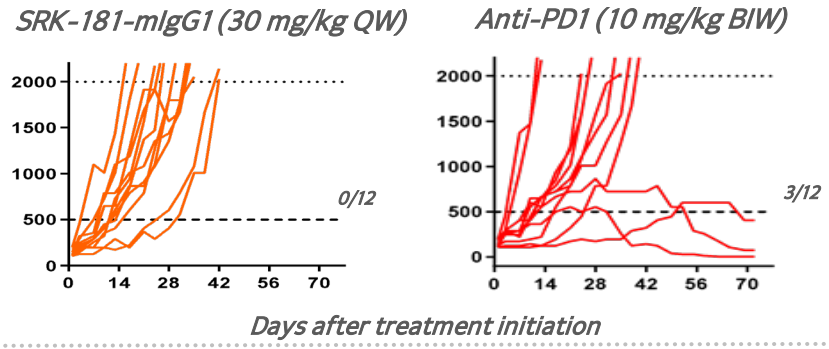
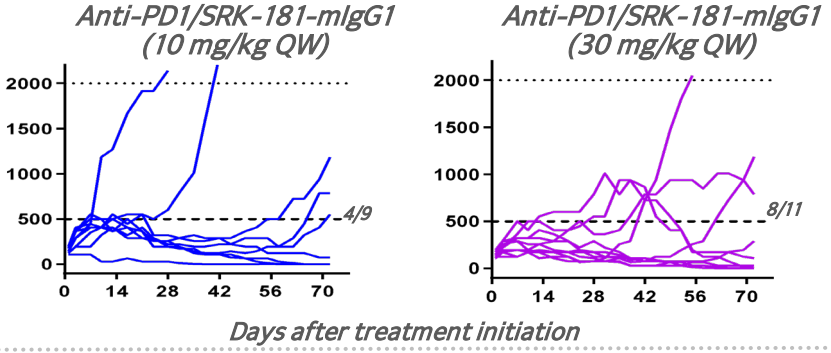
TGFβ1 Blockade with SRK-181-mIgG1 Rendered Preclinical Tumor Models Susceptible to Anti-PD1 Therapy

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

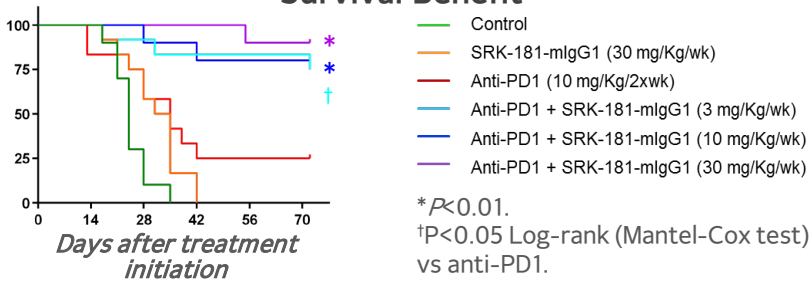
Tumor Regression: Monotherapy



Tumor Regression: Combination Therapy



Survival Benefit



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

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

Preclinical data published in *Science Translational Medicine*. Martin CJ, et al. *Sci Transl Med.* 2020 Mar 25;12(536):eaay8456. https://scholarrock.com/platform/publications.

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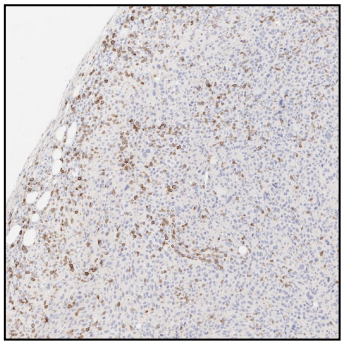
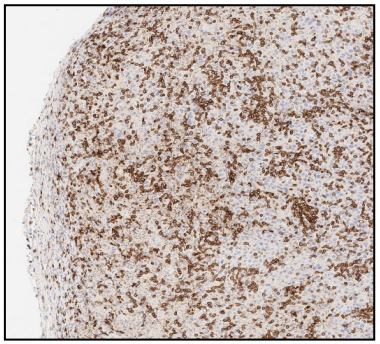
SRK-181-mIgG1 Combination Therapy Enabled Infiltration and Expansion of CD8+ T cells



Anti-PD1



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

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

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

Preclinical data published in *Science Translational Medicine*. Martin CJ, et al. *Sci Transl Med*. 2020 Mar 25;12(536):eaay8456. https://scholarrock.com/platform/publications.

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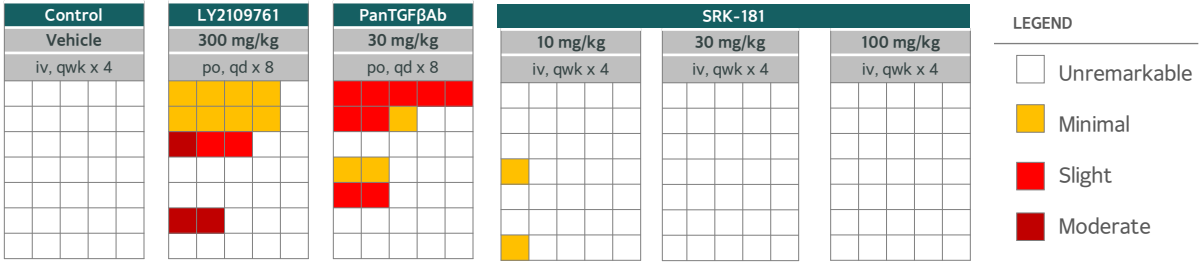
TGFβ1 Isoform Specificity of SRK-181 Improved Preclinical Toxicity Profile

Microscopic observations in heart
Valvulopathy
Atrium—Mixed cell infiltrate
Myocardium—Degeneration/necrosis
Myocardium—Hemorrhage
Myocardium—Miced cell infiltrate, base
Coronary artery—Necrosis with inflammation
Cardiomyocyte—Necrosis/inflammatory cell infiltrate



Repeat dose pilot toxicology study in adult female Sprague Dawley rats:

- Cardiac findings were exhibited in animals dosed with a pan-TGFβ antibody or LY2109761 (inhibitor of ALK5, common TGFβ receptor kinase) as expected based on published data†
- No cardiotoxicities (valvulopathy) were noted with SRK-181
  - NOAEL for SRK-181 was the highest dose evaluated of 100 mg/kg QW

4-week GLP toxicology studies:

- Rats: NOAEL for SRK-181 was up to highest evaluated dose of 200 mg/kg QW
- Non-human primates: NOAEL for SRK-181 was up to highest evaluated dose of 300 mg/kg QW

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

Preclinical data published in *Science Translational Medicine*. Martin CJ, et al. *Sci Transl Med* 2020 Mar 25;12(536): eaay8456.
*Source: Anderton MJ, et al. Induction of heart valve lesions by small-molecule ALK5 inhibitors. *Toxicol Pathol*. 2011;39: 916-924.; and Stauber AJ, et al. Nonclinical safety evaluation of a transforming growth factor β Receptor I kinase inhibitor in Fischer 344 rats and beagle dogs. *J Clin Pract*. 2014: 4:3.

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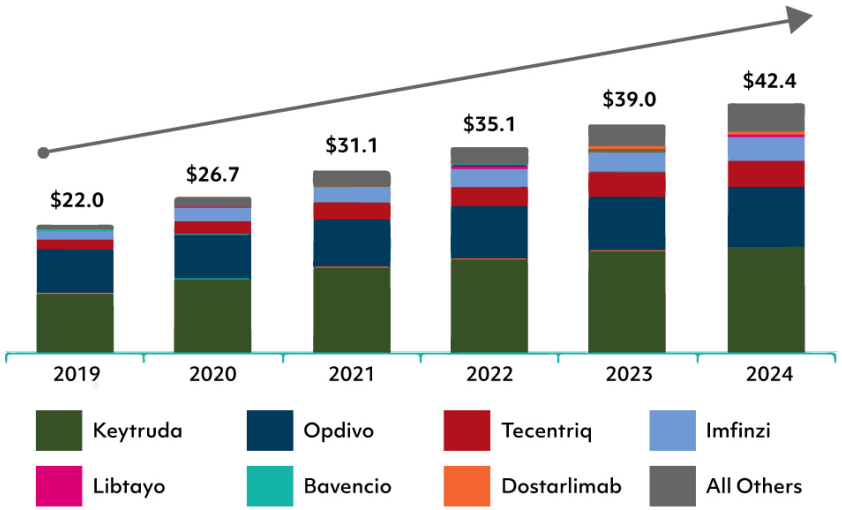
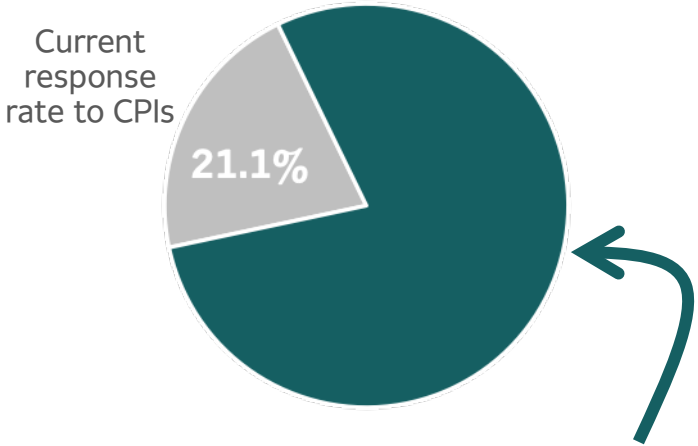
SRK-181 Has Potential to Increase Response to CPIs Through a Clinically Relevant Resistance Pathway

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



…Yet medical need is 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 A, et al. *Oncotarget*. 2018;9:8706-8715.

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DRAGON Phase 1 POC Trial to Evaluate SRK-181's Ability to Overcome Primary Resistance to Checkpoint Inhibitors

Update on dose escalation in 4Q20; clinical response and safety data in 2021

SRK-181:
selective inhibitor of TGFβ1 activation

→

Part A

Part A1:
- SRK-181 as a single agent
- Modified 3+3 dose escalation
- Assess SRK-181 dose range of 80-2400 mg (avg weight 80kg)

Part A2:
- SRK-181 with approved anti-PD-(L)1
- 3+3 dose escalation
- Treat with same anti-PD-(L)1 as previously given with no response

→

Part B

- SRK-181 in combo with approved anti-PD-(L)1
- Multiple parallel cohorts – each will enroll up to 40 patients
- Target indications expected to include NSCLC, urothelial carcinoma, melanoma, amongst other solid tumor types
- Treat with same anti-PD-(L)1 as previously given with no response
- Most recent dose of anti-PD-(L)1 therapy ≤6 months prior to enrollment

- Open-label, dose escalation, and dose expansion clinical trial
- Evaluate the efficacy, safety/tolerability, and PK/PD of SRK-181 in combination with approved anti-PD-(L)1 therapy
- Patients with locally advanced or metastatic solid tumors that exhibit primary resistance to anti-PD(L)1 therapy
- Lack of response characterized as stable or progressive disease following ≥3 cycles of anti-PD-(L)1 therapy either alone or in combination with chemotherapy

NCT04291079 on www.clinicaltrials.gov.

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

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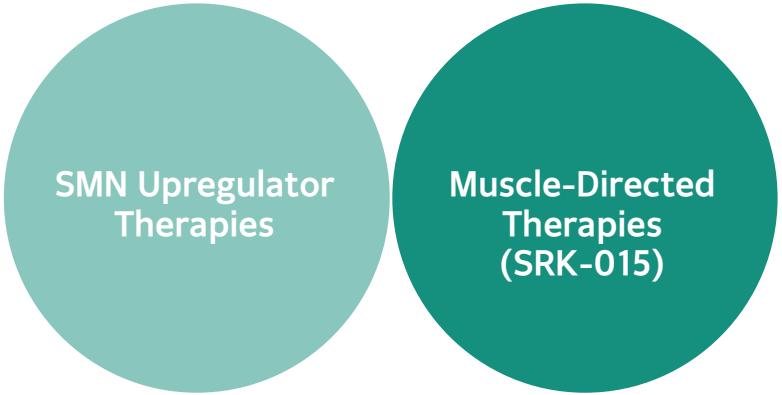
Significant Unmet Need Remains Despite Current Therapies

Muscle-directed therapies needed to complement disease-stabilizing benefits of SMN upregulators*



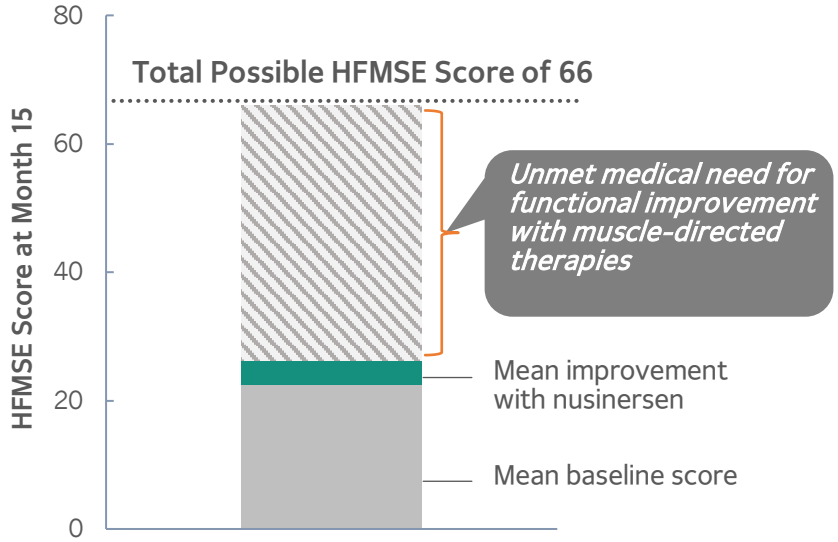
Address SMN deficiency to prevent further motor neuron deterioration

Act directly on muscle with aim to improve motor function

SMN = survival motor neuron.
**Also referred to as SMN correctors.*

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

Source: Mercuri E, et.al. Nusinersen versus sham control in later-onset spinal muscular atrophy. *N Engl J Med.* 2018;378:625-635.

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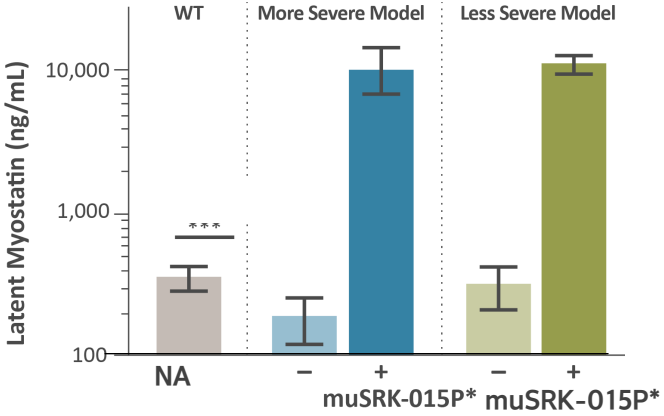
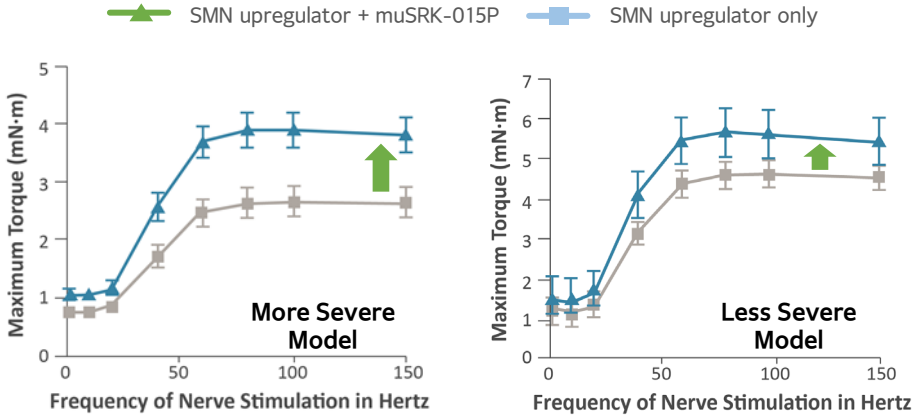
Treatment of SMNΔ7 Mouse Models Show Improvement in Muscle Strength



- 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 in *Human Molecular Genetics* (first published online November 27, 2018). Long K, et al. Specific inhibition of myostatin activation is beneficial in mouse models of SMA therapy. *Hum Mol Genet.* 2019;28(7):1076-1089. https://scholarrock.com/platform/publications/.

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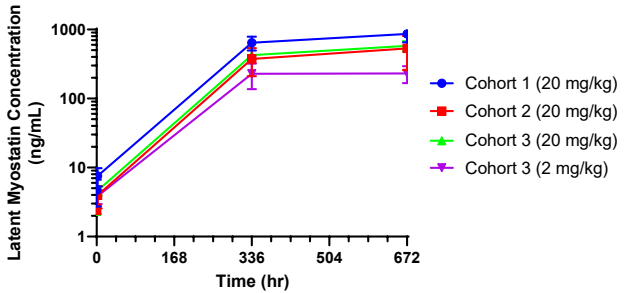
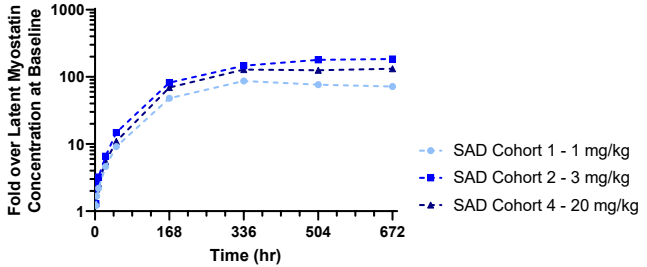
Preliminary TOPAZ Biomarker Data Provide First Demonstration of Target Engagement in Patients with SMA

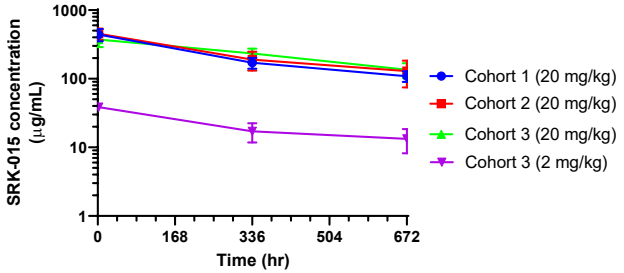
Latent Myostatin Change Over Baseline in SRK-015 TOPAZ Trial



Latent Myostatin Change Over Baseline in Phase 1 HV Trial



Preliminary TOPAZ Phase 2 Pharmacokinetic (PK) Data



Robust Target Engagement Observed

- ~100-fold increases in serum latent myostatin levels following single 20 mg/kg dose in all cohorts of TOPAZ
- 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

Preliminary PK/PD results include data from 29 patients (12 in Cohort 1, 8 in Cohort 2, and 9 in Cohort 3).
Source: Poster presentation at the MDA Clinical and Scientific Conference (March 2020). https://scholarrock.com/platform/publications/.

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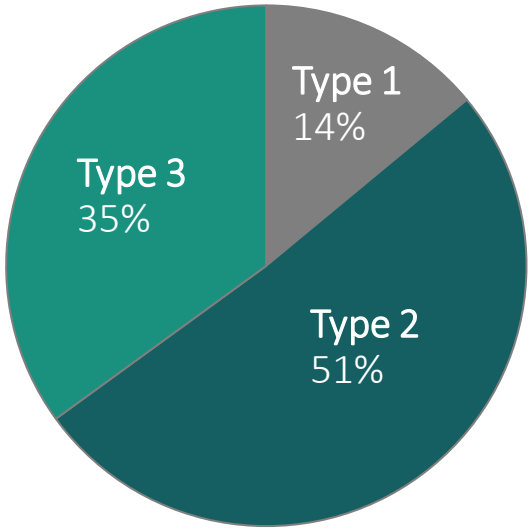
SRK-015 Opportunity in Spinal Muscular Atrophy

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

Relative Prevalence Among Patients Living With SMA



Type 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 C, et al. *Orphanet J Rare Dis.* 2017;12:175.

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TOPAZ

SRK-015 Phase 2 Trial Design

Interim Efficacy and Safety Results 4Q20; Top-line 12-Month Data 1H21

	Cohort 1	Cohort 2	Cohort 3
Design	• N= 23*; 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

Each cohort evaluates a subpopulation that is sizable and has substantial unmet medical need

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.
**Baseline demographics presented as part of AAN virtual platform (May 2020).* https://scholarrock.com/platform/publications/.

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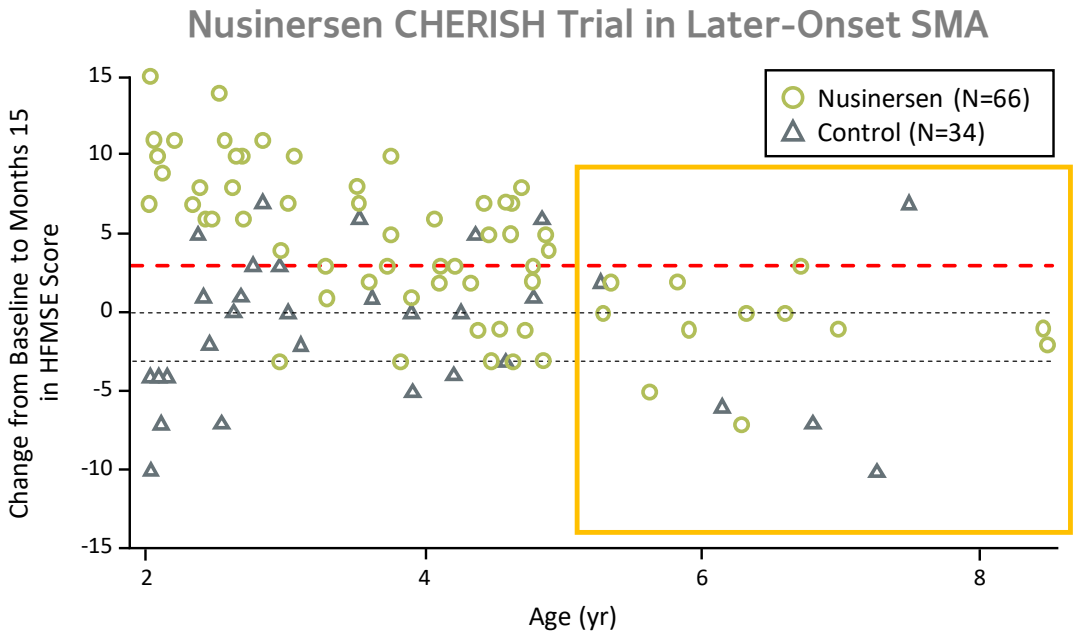
Later-Onset SMA: High Unmet Need to Improve Motor Function



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 rare (<15% of patients) even with nusinersen treatment

3-point improvement in a patient is considered clinically meaningful and rare to observe

Source: Mercuri E, et.al. Nusinersen versus sham control in later-onset spinal muscular atrophy. *N Engl J Med.* 2018;378:625-635.

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Building a Differentiated Pipeline Through a Highly Productive Platform

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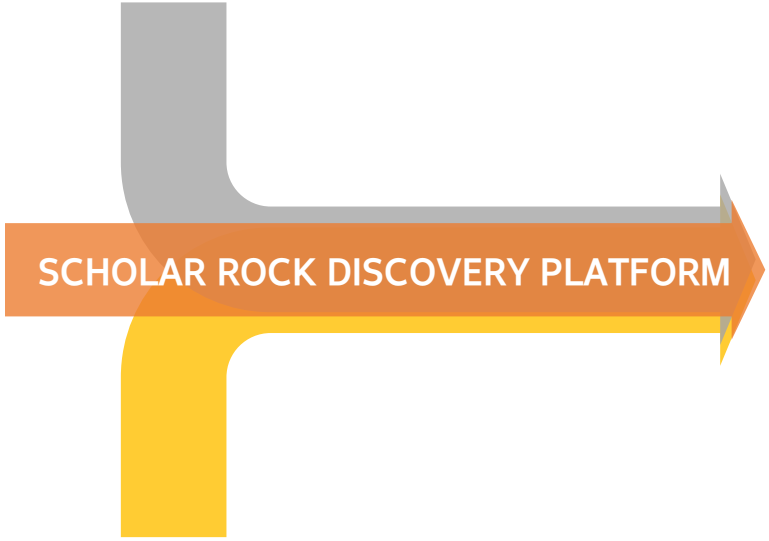
Proprietary Platform to Target Latent Growth Factor Activation

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

- Oncology
- Neuromuscular Disorders
- Fibrosis
- Anemia

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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

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

Gilead Sciences, Inc.

- 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.*

Upcoming Key R&D Milestones

Oncology

- Update on dose escalation from SRK-181 DRAGON Phase 1 POC trial in 4Q20*
  - SRK-181 as a single-agent as well as in combination with approved anti-PD-(L)1 therapy
- Clinical response and safety data from SRK-181 Phase 1 POC trial in 2021*
- Continue to advance active discovery programs for context-dependent inhibition of TGFβ1

Spinal Muscular Atrophy (SMA)

- Interim efficacy and safety results from SRK-015 TOPAZ Phase 2 trial in 4Q20*
  - Patients from 3 cohorts progressed through at least 6 months of the treatment period
- Top-line results (12 months) from SRK-015 TOPAZ trial in 1H21*
  - Patients are eligible to continue treatment for additional 12 months
- Identification of second indication for SRK-015 in 2020

Fibrosis

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

Anemias

- Nominate product candidate in RGMc program in 2020

*Timing of data read-outs may be impacted by the COVID-19 pandemic.

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Appendix

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

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 product candidates, SRK-015 and SRK-181, in clinical development
- 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 of growth factors and beyond

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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 toxicity often seen with traditional, less-selective approaches*

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

Commenced dosing of DRAGON Phase 1 POC trial for SRK-181 in 2Q20

- Update on dose escalation in 4Q20
- Clinical response and safety data in 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 https://scholarrock.com/platform/publications/

*Source: Carretero-Gonzalez A, et al. *Oncotarget*. 2018;9:8706-8715.

†Source: Mariathasan S, et.al TGFβ attenuates tumour response to PD-L1 blockade by contributing to exclusion of T cells. *Nature*. 2018 Feb 22;554(7693):544-548. https://doi.org/10.1038/nature25501.

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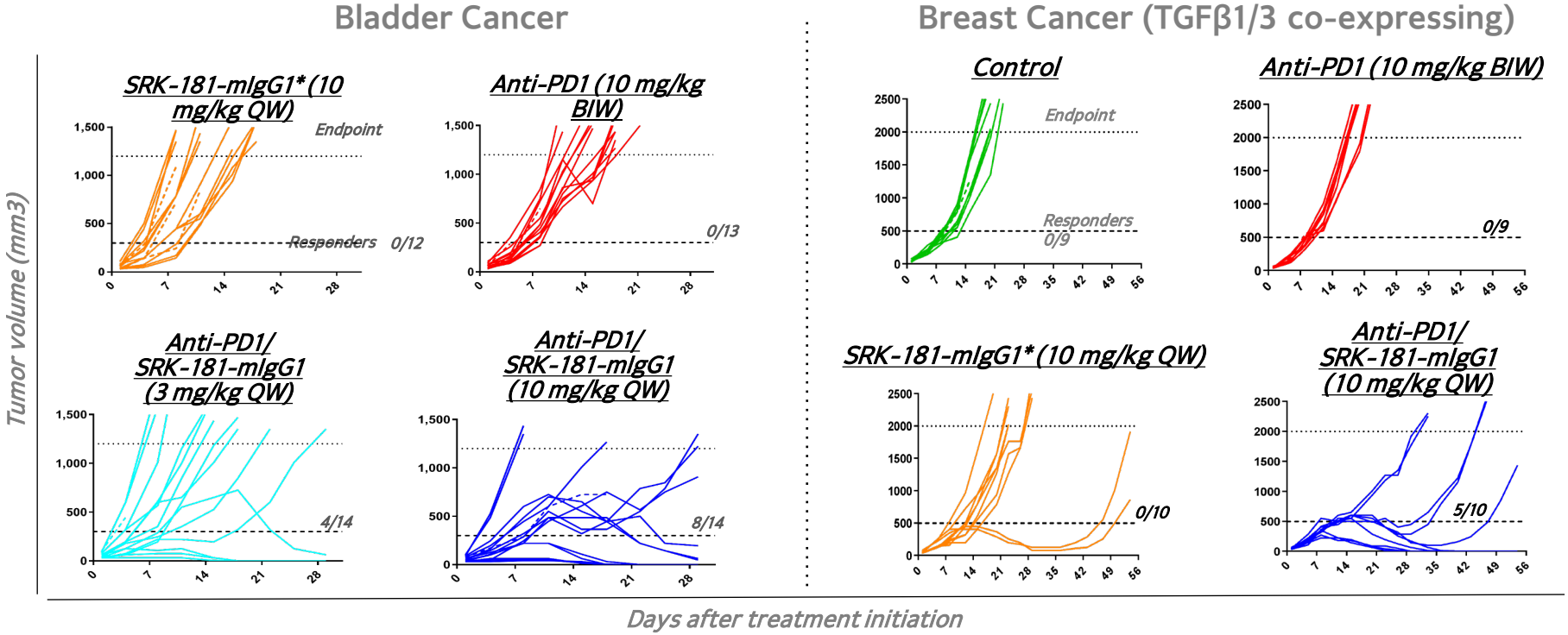
TGFβ1 Blockade with SRK-181-mIgG1 Rendered Preclinical Tumor Models Susceptible to Anti-PD1 Therapy



Preclinical data published in *Science Translational Medicine*. Martin CJ, et al. *Sci Transl Med*. 2020 Mar 25;12(536):eaay8456. https://scholarrock.com/platform/publications/.

**SRK-181-mIgG1 is the murine version of SRK-181; responder defined as tumor size <25% endpoint volume at study end.*

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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)*

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: interim efficacy and safety data in 4Q20

- Top-line 12-month analysis anticipated 1H21

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 poster presentation at the MDA Clinical and Scientific Conference (March 2020) available at https://scholarrock.com/platform/publications/.

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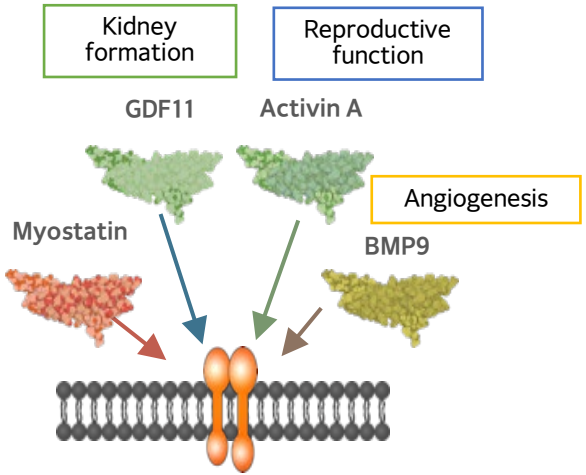
Traditional Approaches Can Raise Significant Safety Concerns

Traditional Approaches

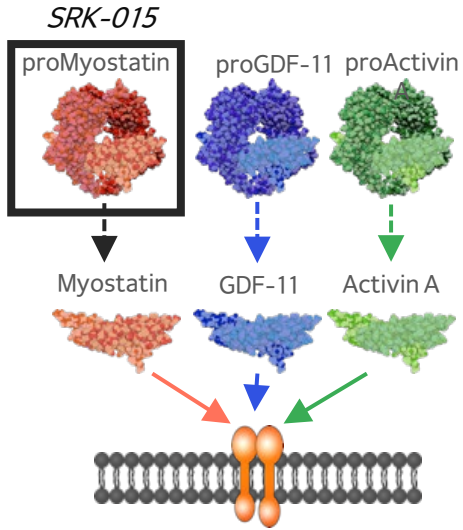
Previous myostatin inhibitors lack sensitivity

- 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



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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*

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, et al. TGF-β1 signaling and tissue fibrosis. *Cold Spring Harb Perspect Biol*. 2018 Apr 2;10(4): a022293.

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