SCHOLAR ROCK

From New Insights to New Medicines

March 2019

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2018: Transformative Year for Scholar Rock

Established Strong Financial Foundation

- Transitioned to public company with successful $86M IPO

Transitioned to Clinical-Stage Company

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

Executed Strategic Collaboration

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

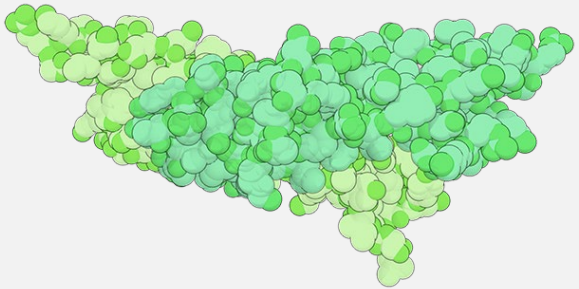
Advanced Innovative Pipeline

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

Nature's Growth Factor Activation Machinery

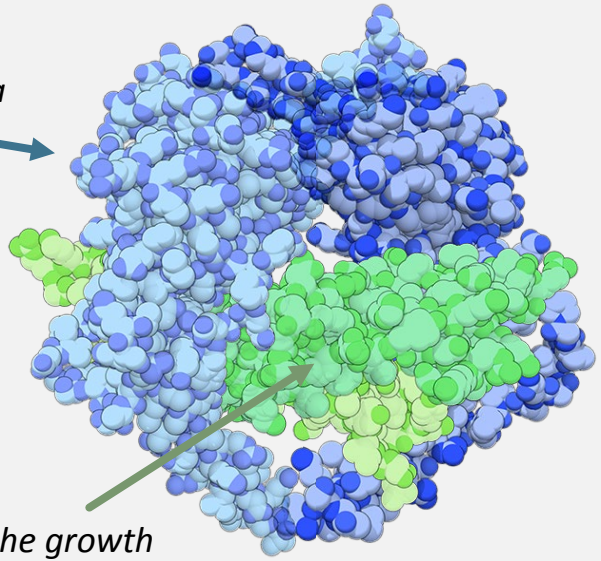
Mature TGFβ1

Active form of the growth factor



TGFβ1 Precursor Complex

TGFβ1 propeptide which forms a "cage"



Cage renders the growth factor inactive or "latent"

TGFβ Superfamily: More than 30 Related Growth Factors that Mediate Diverse Biological Processes

Shi, M., et al. Nature 474, 7351, 343-9 (2011)

Robust Pipeline Portfolio

	Program / Target	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						Initiate Phase 2 POC Trial in 1Q19
	SRK-015	Myostatin-Related Disorders						Identify Next Indication in 2020
Latent TGFβ	SRK-181 (Context-Independent Latent TGFβ1)	Oncology/Immuno-oncology						Initiate Phase 1 Trial Mid-2020
	Context-Dependent Latent TGFβ1 / Immune Cell	Oncology/Immuno-oncology						
BMP6	BMP6 Signaling Pathway	Anemia						
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	

SRK-015: Inhibitor of Myostatin Activation

Potential First Muscle-Directed Therapy for SMA

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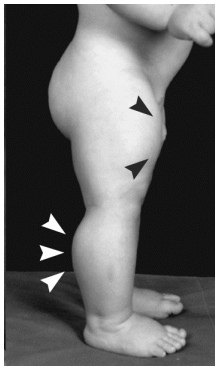
SRK-015: Highly Specific Inhibitor of Latent Myostatin



- 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

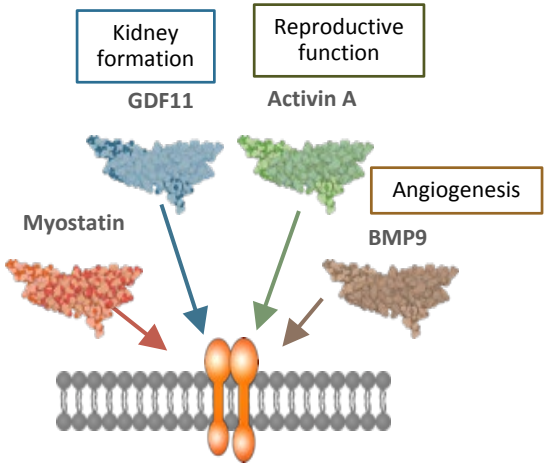
Differentiated approach with SRK-015:

- ***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)***

Traditional Approaches Can Raise Significant Safety Concerns

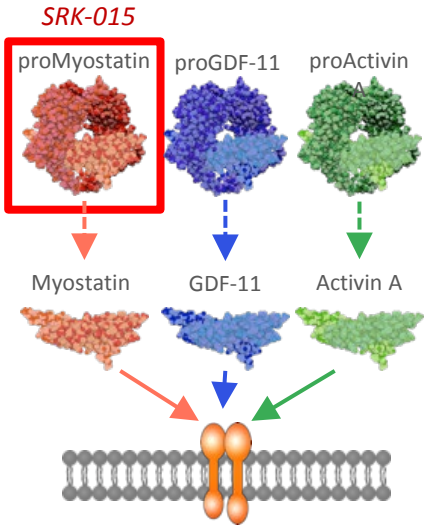
Traditional Approaches Lack Selectivity

- 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

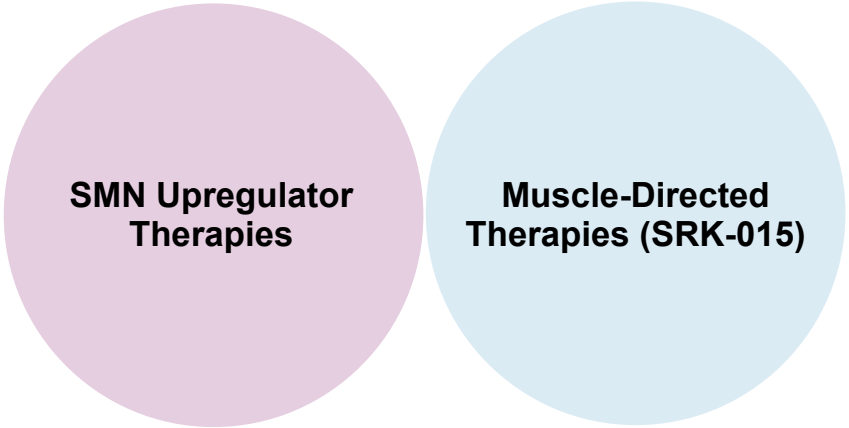
Exquisite Selectivity By Targeting Precursor Form of Myostatin



SRK-015: Aligning Therapeutic Approach with Myostatin Biology

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

Significant Unmet Need Remains Despite Current Therapies



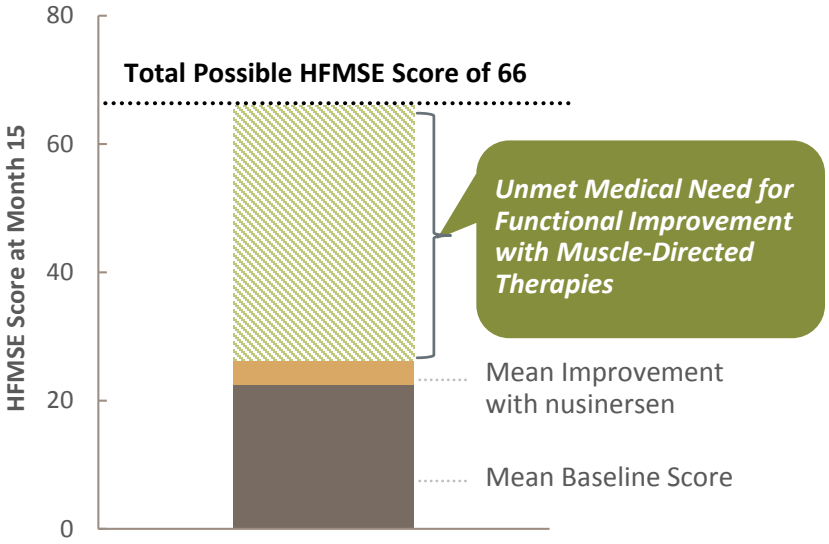
Address SMN deficiency to prevent further motor neuron deterioration

Act directly on muscle with aim to improve functional performance

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

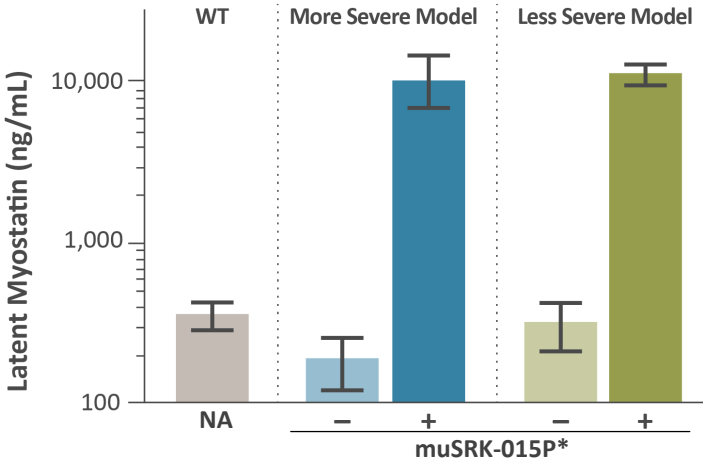
Muscle Function in SMA (Human)

Hammersmith Functional Motor Scale Expanded (HFMSE)

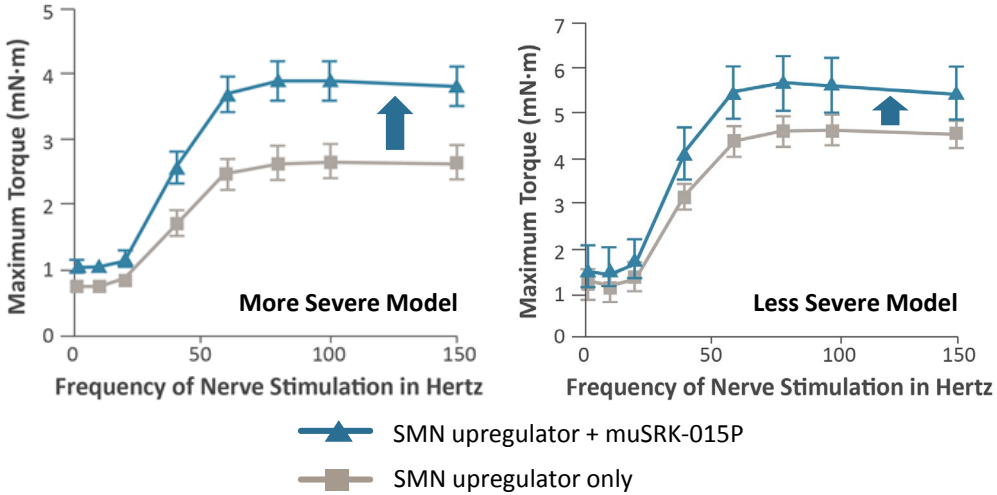


Mean improvement in HFMSE score experienced by patients with later-onset SMA in the Phase 3 CHERISH clinical trial of nusinersen

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

Phase 1 Interim Safety Results Support Advancing to Phase 2 Trial

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

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

PK Data Support Infrequent Dosing

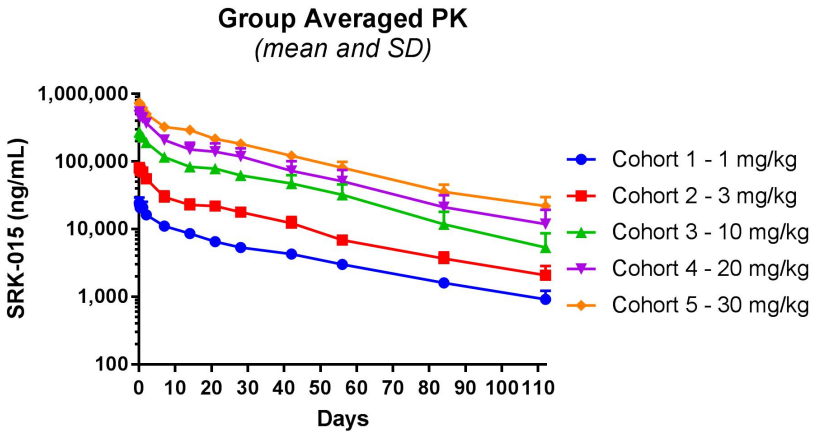
Displayed Well-Behaved PK Profile

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

Half-Life Supports Infrequent Dosing

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

Pharmacokinetic (PK) Data from Single-Ascending Dose



PD Data Demonstrate Robust and Sustained Target Engagement

Robust Target Engagement Observed

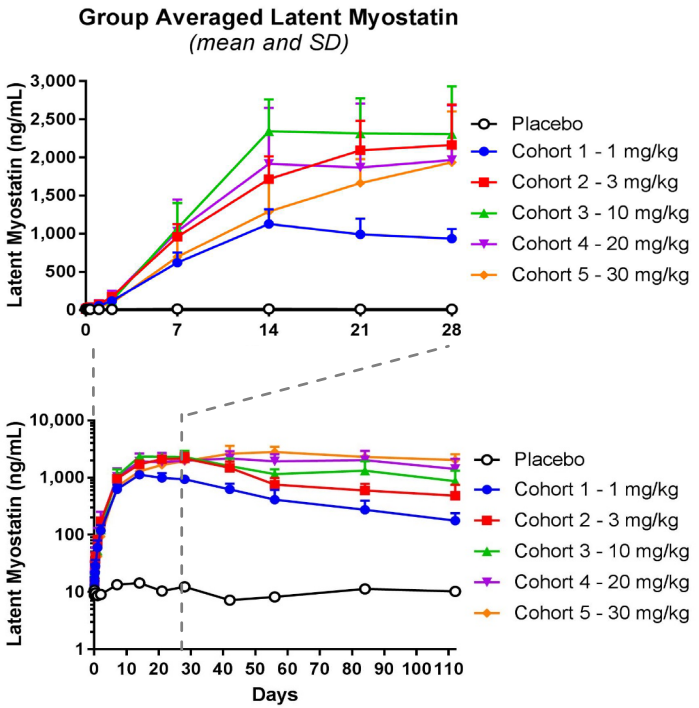
- Marked increases in serum concentrations of latent myostatin following a single dose of SRK-015
- No meaningful change observed with placebo

Evidence Supports Durable Target Saturation

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

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

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



SRK-015 Target Profile in SMA

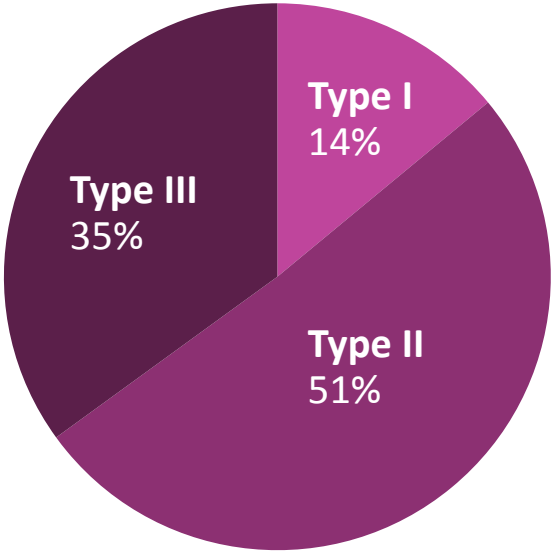
GOALS	EVIDENCE TO DATE
Effectively increase motor function to drive clinically meaningful outcomes	✓ Translational/preclinical data support myostatin as a drug target in SMA ✓ Preclinical data demonstrate potential for substantial increases in muscle strength ✓ Interim Phase 1 PD data demonstrate SRK-015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

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

SRK-015 Opportunity in Spinal Muscular Atrophy

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

Relative Prevalence Among Patients Living With SMA



Type I:

- Infant-onset; often fatal

Type II and non-ambulatory type III:

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

Ambulatory type III:

- Limited mobility and substantial morbidity

Type IV:

- Population not well-characterized

Focus of Phase 2 Trial

Potential to use SRK-015 in conjunction with SMN upregulators

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

Lally et al, Orphanet Journal of Rare Diseases, 2017

Overview of Phase 2 TOPAZ Trial in SMA

Design

- 3 cohorts; total of 50-60 patients
- 12-month treatment period
- SRK-015 IV every 4 weeks (Q4W)

Subjects

- **Type 3 ambulatory SMA** (monotherapy or in conjunction with approved SMN upregulator)
- **Type 2 and Type 3 non-ambulatory SMA** (in conjunction with approved SMN upregulator)
- **Type 2 SMA very young children** (in conjunction with approved SMN upregulator)

Key Objectives

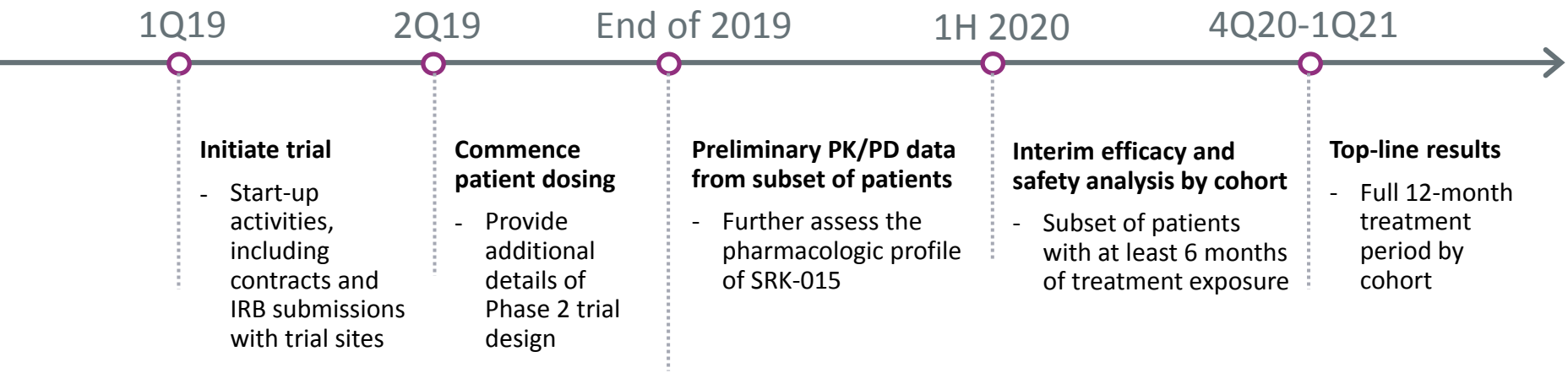
- HFMSE (non-ambulatory SMA)
- RHS (ambulatory SMA)
- Safety

Timeline

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

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

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



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

TGFβ1: Significant Opportunities in Oncology/Immuno-oncology and Fibrosis

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TGFβ1 Plays Central Role in Multiple Diseases with Unmet Need

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Oncology

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

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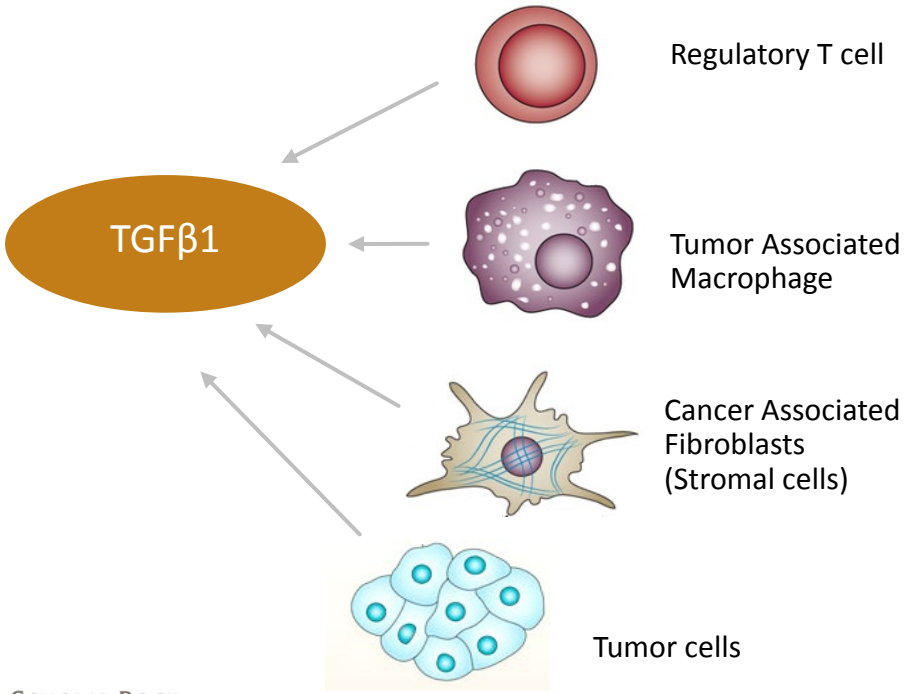
Fibrosis

GILEAD

Inhibition of TGFβ1: Multipronged Approach for Immuno-Oncology

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



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

doi:10.1038/nature25501

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

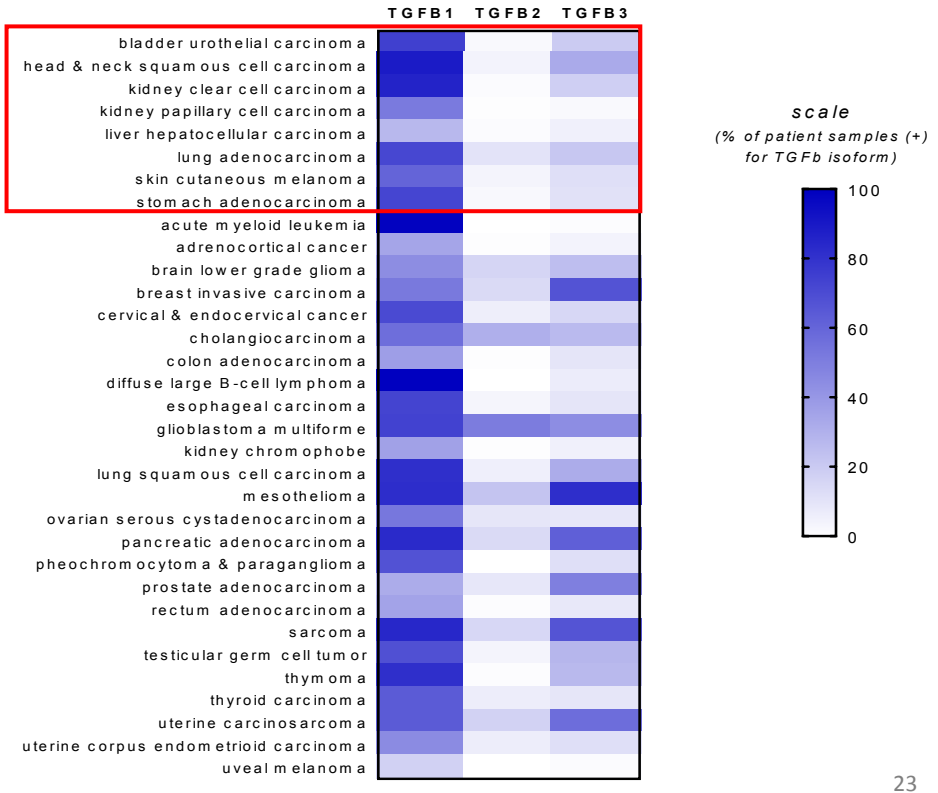
Sanjeev Mariathasan[1]*, Shannon J. Turley[1]*, Dorothee Nickles[1]*, Alessandra Castiglioni[1], Kobe Yuen[1], Yulei Wang[1], Edward E. Kadel III[1], Hartmut Koeppen[1], Jillian L. Astarita[1], Rafael Cubas[1], Suchit Jhunjhunwala[1], Romain Banchereau[1], Yagai Yang[1], Yinghui Guan[1], Cecile Chalouni[1], 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]

- Pathway analysis points to TGFβ1 as major determinant of resistance to anti-PD-L1 (atezolizumab)
- TGFβ1 creates ‘immune excluded’ tumor microenvironment
- Anti-TGFβ antibody enhances anti-PDL1 treatment response in syngeneic EMT-6 tumor model

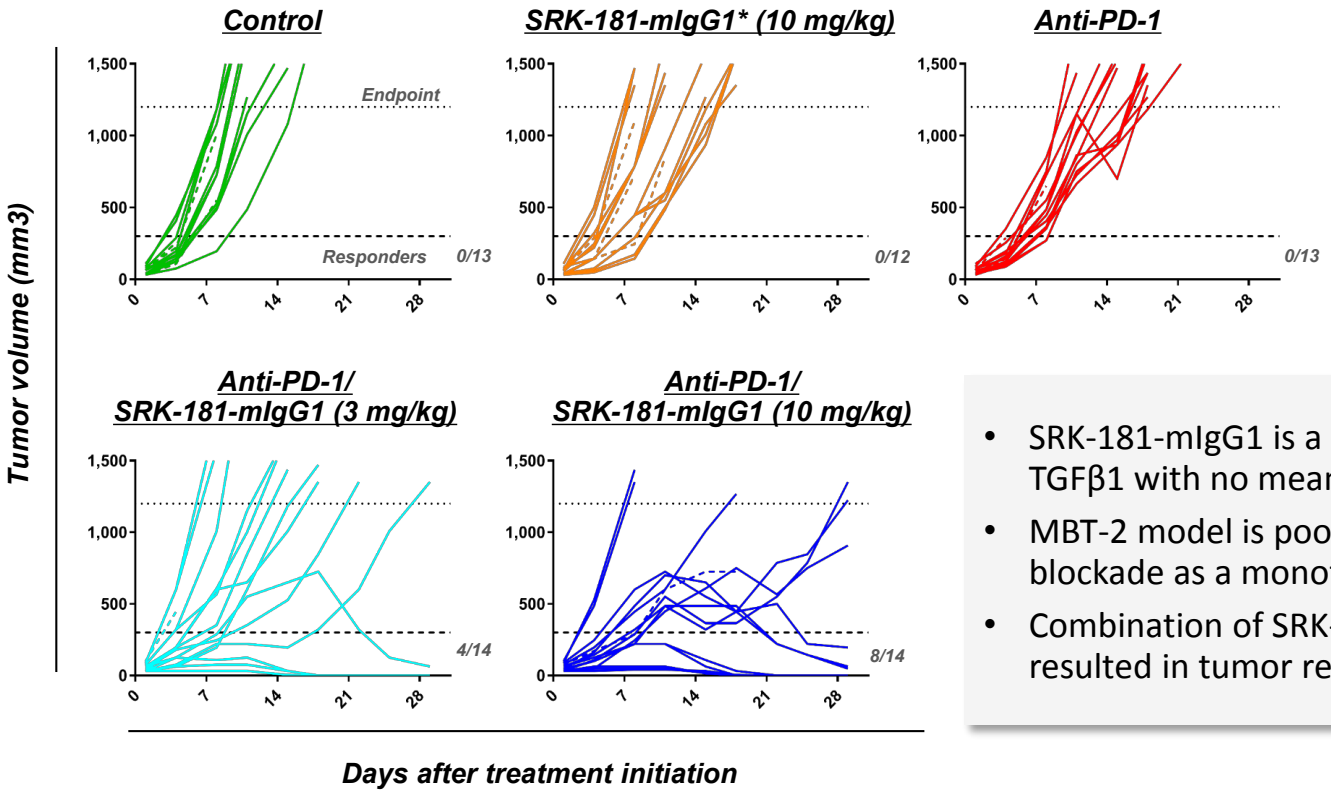
TGFβ1 is the Predominant Isoform in Most Human Tumors

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



TGFβ1 Blockade with SRK-181-mIgG1 Renders Preclinical Bladder Tumors Susceptible to Anti-PD1 Therapy



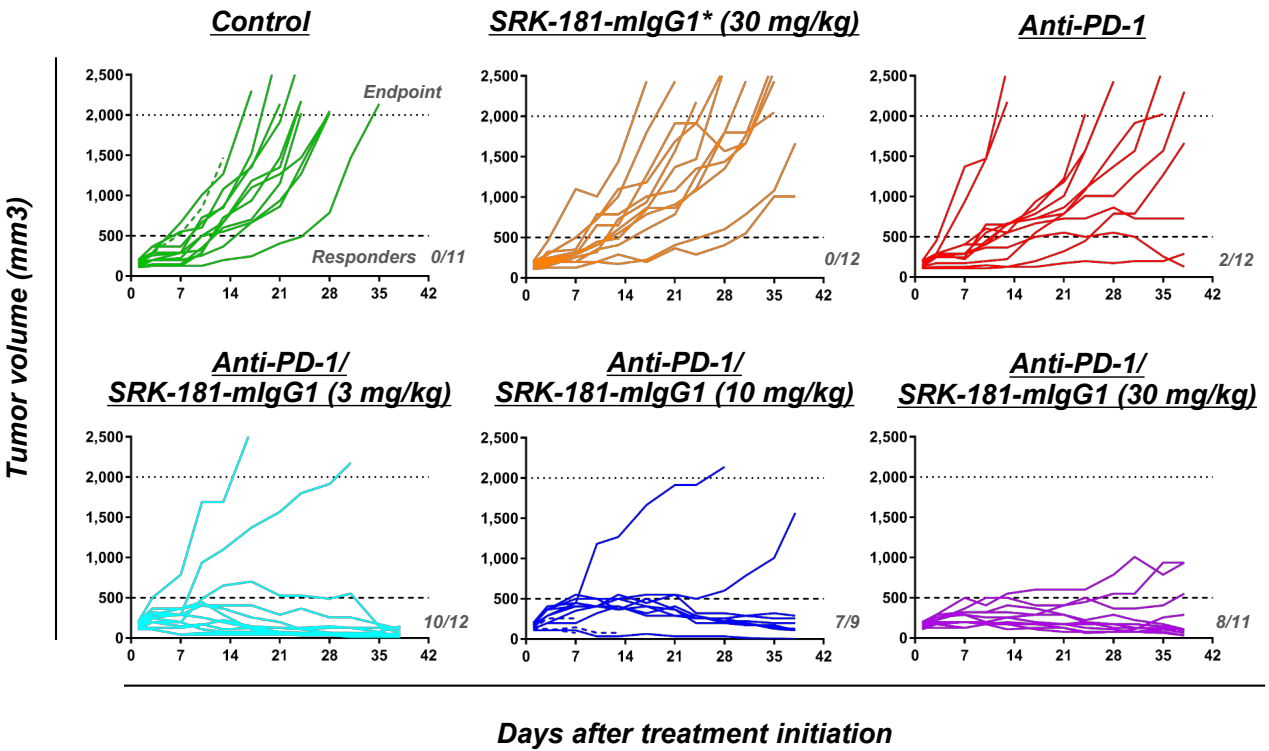
Dosing initiated when tumors reached 30-80 mm^3

Treatment with RMP1-14 anti-PD1 (5mg/kg twice weekly) and/or SRK-181-mIgG1 (once weekly at indicated dose)

- SRK-181-mIgG1 is a highly specific inhibitor of TGFβ1 with no meaningful binding to TGFβ2/TGFβ3
- MBT-2 model is poorly responsive to PD-1 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 Society for Immunotherapy of Cancer (SITC) 33rd Annual Meeting (Nov 2018)
* SRK-181-mIgG1 was formerly referred to as SRTβ1-Ab3

Anti-TGFβ1 Combination with PD-1 Blockade is Effective in Preclinical Melanoma Model

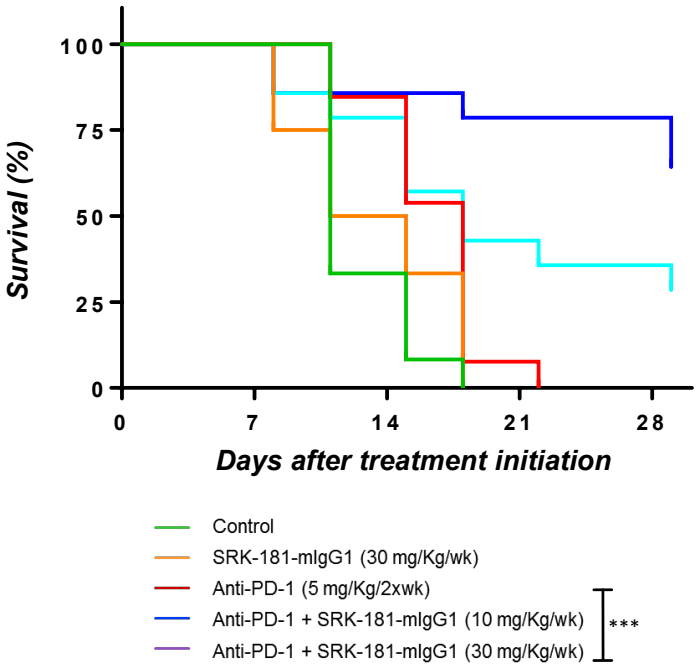


- Cloudman S91 model is poorly responsive to PD-1 blockade as a monotherapy
- Combination of SRK-181-mIgG1 and anti-PD1 resulted in tumor regression or tumor control

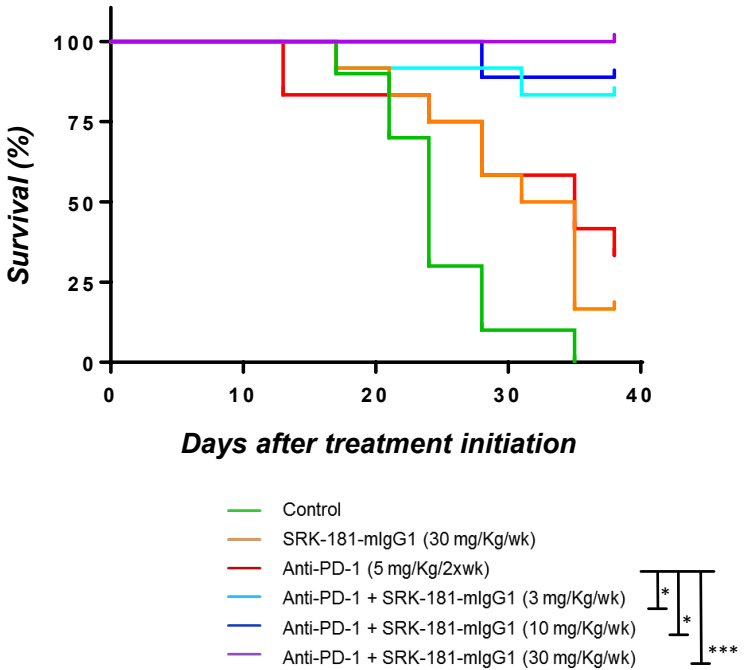
Interim preclinical data shown above as presented at Society for Immunotherapy of Cancer (SITC) 33rd Annual Meeting (Nov 2018)
* SRK-181-mIgG1 was formerly referred to as SRTβ1-Ab3

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

MBT-2 Bladder Cancer Model



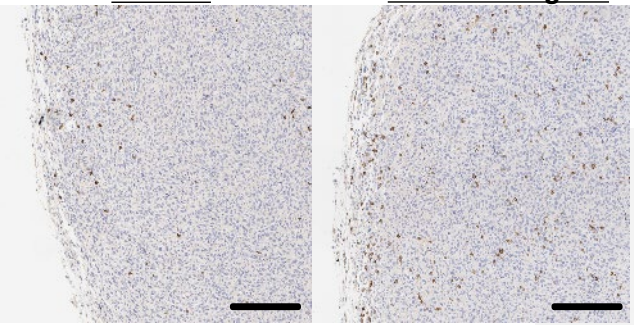
Cloudman S91 Melanoma Model

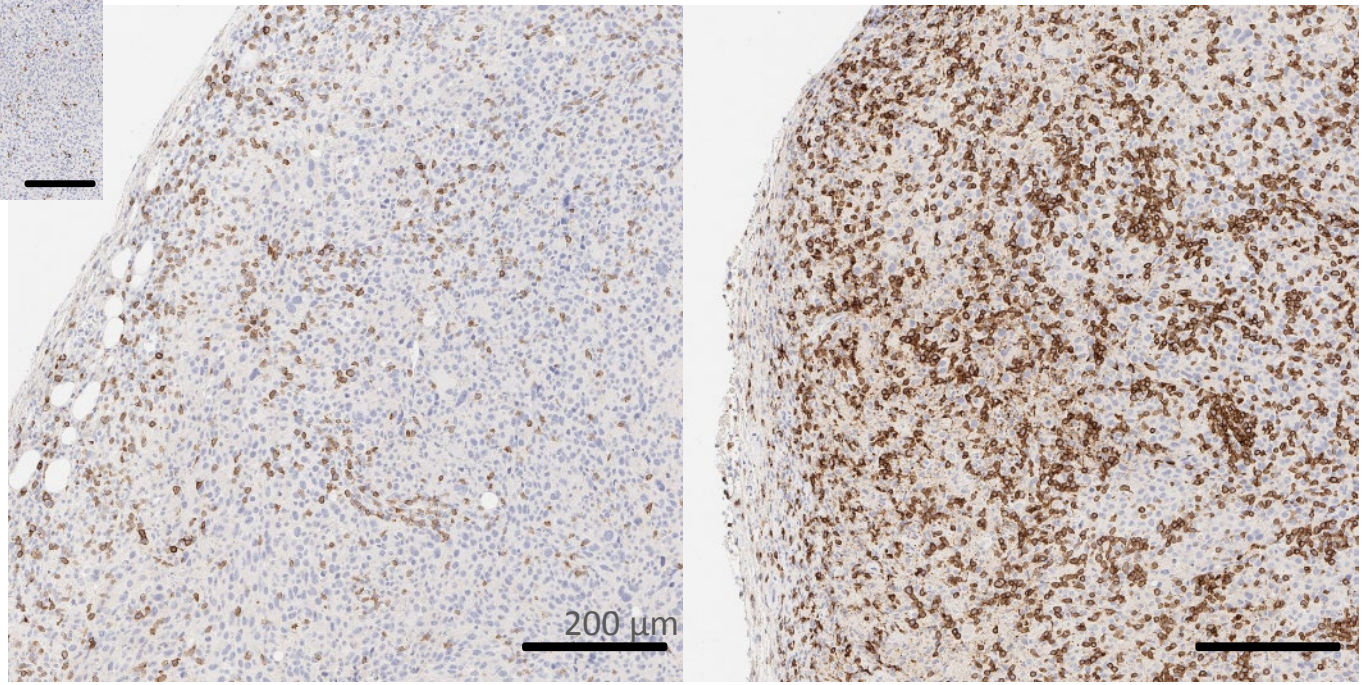


* P<0.05 Log-rank (Mantel-Cox test)
*** P<0.001

Preclinical data shown above as presented at Society for Immunotherapy of Cancer (SITC) 33rd Annual Meeting (Nov 2018)
** SRK-181-mIgG1 was formerly referred to as SRTβ1-Ab3

SRK-181-mIgG1 Combination Therapy Enables Infiltration and Expansion of CD8+ T cells in Preclinical Tumor Model





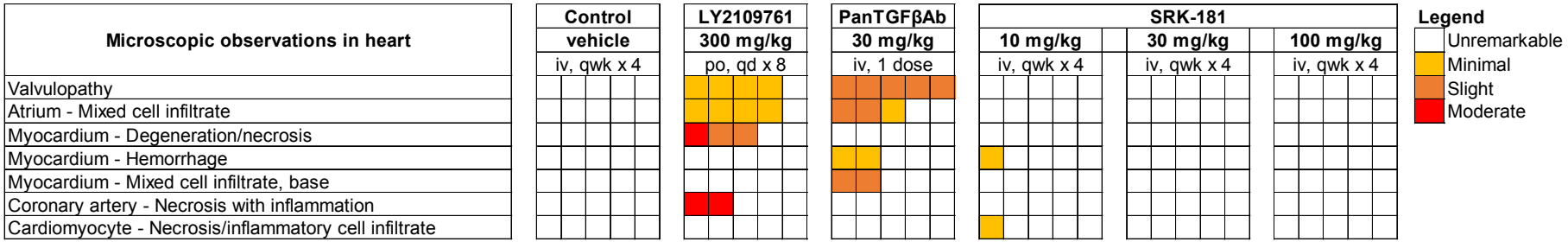
IHC of MBT-2 tumors at day 10

- Marked increase in frequency of CD8+ T cells within the tumor
- Consistent with flow cytometry data at day 13

Preclinical data shown above as presented at Society for Immunotherapy of Cancer (SITC) 33rd Annual Meeting (Nov 2018)
* SRK-181-mIgG1 was formerly referred to as SRTβ1-Ab3

TGFβ1 Isoform Specificity of SRK-181 Results in 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)

SRK-181: Advancing Development for Treatment of Cancers Resistant to Checkpoint Blockade Therapies (CBTs)

SRK-181 is a fully human antibody designed to bind to, and prevent the activation of, latent TGFβ1 with high affinity and high selectivity

TGFβ signaling	→	Implicated as a culprit in primary resistance to CBTs
Translational data analyses	→	TGFβ1 expression is prominent in many human tumor types for which CBTs is approved or showed clinical activity
Clinical correlation and preclinical model data	→	TGFβ1 excludes effector cell entry into the tumor and limits immune system access to tumor cells
Preclinical studies in syngeneic mouse tumor models resistant to CBT	→	Combination of SRK-181-mIgG1* with anti-PD1 led to tumor regression/control and significant survival benefit
28-day pilot toxicology study in adult rats	→	SRK-181 showed no observed drug-related toxicity up to a weekly dose of 100 mg/kg for 4 weeks

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

Highlights of Strategic Fibrosis Collaboration with Gilead

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Responsible for antibody discovery and preclinical research thru product candidate nomination for three TGFβ programs

Retains exclusive worldwide rights to develop certain TGFβ antibodies for oncology and cancer immunotherapy

Collaborating to Develop Innovative Therapies for Fibrotic Diseases

GILEAD

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

Upfront cash and equity investment:
$80 million*

One-time preclinical milestone:
$25 million

Additional development, regulatory, and commercial 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*

Upcoming Key R&D Milestones

SRK-015

- Initiate Phase 2 SMA proof-of-concept trial by the end of 1Q19
- Commence patient dosing in Phase 2 SMA proof-of-concept trial in 2Q19
- Present full Phase 1 results at a scientific conference in 2019
- Continue to evaluate selective inhibitors of myostatin activation in multiple disease models
- Identify next indication in 2020
- Announce Phase 2 trial read-outs:
  - Preliminary PK/PD analysis by end of 2019
  - Interim efficacy and safety analysis at 6 months in 1H20
  - Top-line results of 12-month treatment period 4Q20-1Q21

TGFβ1 Inhibitor

- Advance cancer immunotherapy product candidate, SRK-181, into a Phase 1 trial mid-2020
- Continue to advance active discovery programs for context-dependent inhibition of TGFβ1
- Conduct fibrosis discovery and preclinical studies in partnership with Gilead

Cash, cash equivalents, and marketable securities at December 31, 2018: ~$176 million
Sufficient to fund operations into 2021

Building Value in All Dimensions

Leveraging Strong Financial Foundation

Advancing Clinical Development

Executing Strategic Collaboration

Growing Innovative Pipeline