miRagen

Restoring Biological Harmony for Patients with Debilitating Disease

miRagen Therapeutics

NASDAQ: MGEN

Oppenheimer 28th Annual Healthcare Conference

March 20, 2018

Cautionary Note Regarding Forward-Looking Statements

This presentation contains forward-looking statements relating to Miragen Therapeutics, Inc., including statements about our plans to obtain funding, develop and commercialize our therapeutic candidates, our planned clinical trials, the timing of and our ability to obtain and maintain regulatory approvals for our therapeutic candidates, the clinical utility of our therapeutic candidates and our intellectual property position. You can identify forward-looking statements by the use of forward-looking terminology including "believes," "expects," "may," "will," "should," "seeks," "intends," "plans," "pro forma," "estimates," or "anticipates" or the negative of these words and phrases or other variations of these words and phrases or comparable terminology. All statements other than statements of historical fact are statements that could be deemed forward-looking statements. These statements involve substantial known and unknown risks, uncertainties and other factors that may cause our actual results, levels of activity, performance or achievements to be materially different from the information expressed or implied by these forward-looking statements. These forward-looking statements should not be relied upon as predictions of future events as we cannot assure you that the events or circumstances reflected in these statements will be achieved or will occur. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements that we make due to a number of important factors, including those risks discussed in "Risk Factors" and elsewhere in our Annual Report on Form 10-K for the year ended December 31, 2017 and our other reports filed with the U.S. Securities and Exchange Commission. The forward-looking statements in this presentation represent our views as of the date of this presentation. We anticipate that subsequent events and developments will cause our views to change. However, while we may elect to update these forward-looking statements at some point in the future, we have no current intention of doing so except to the extent required by applicable law. You should, therefore, not rely on these forward-looking statements as representing our views as of any date subsequent to the date of this presentation.

This presentation also contains estimates and other statistical data made by independent parties and by us relating to market size and other data about our industry. This data involves a number of assumptions and limitations, and you are cautioned not to give undue weight to such estimates. In addition, projections, assumptions and estimates of our future performance and the future performance of the markets in which we operate are necessarily subject to a high degree of uncertainty and risk.

miRagen Therapeutics Highlights

- A leader in microRNA-targeted drug discovery and development with next generation nucleic acid therapeutics platform
- Cobomarsen in blood cancer
  - Human Phase 1 clinical proof-of-concept achieved in 2017 in Cutaneous T-Cell Lymphoma (CTCL)
  - Anticipate commencing Phase 2 clinical trial in CTCL in second half of 2018
  - Anticipate data for Phase 1 clinical trial in the following blood cancers in 2018:
    - Adult T-Cell Lymphoma/Leukemia (ATLL)
    - Diffuse large-B cell lymphoma (DLBCL)
    - Chronic lymphocytic leukemia (CLL)
- MRG-201 in pathological fibrosis
  - Mechanistic proof-of-concept in 2017 Phase 1 clinical trial
  - Anticipate commencing Phase 2a clinical trial in cutaneous fibrosis in 2018
- MRG-110 in cardiovascular disease
  - Anticipate commencing two Phase 1 clinical trials in first half of 2018
  - Development funded by Servier; miRagen retains commercial rights in the United States and Japan

Experienced Executive Leadership Team

William S. Marshall, Ph.D.
President & Chief Executive Officer

ThermoFisher SCIENTIFIC | DHARMACON RNA TECHNOLOGIES | AMGEN

Adam Levy
Chief Business Officer

WEDBUSH | Merrill Lynch | Jefferies

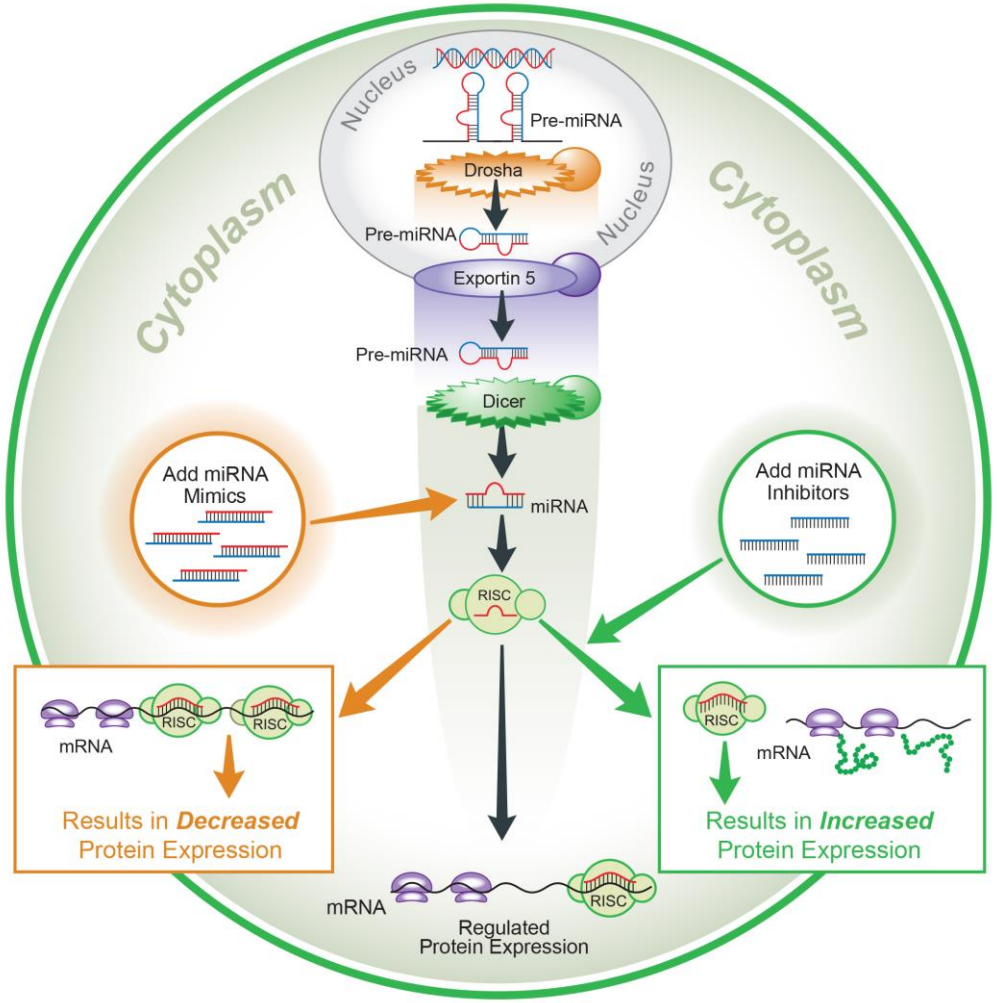
Jason A. Leverone, C.P.A.
Chief Financial Officer

Replidyne | CreekPath SYSTEMS | ARTHUR ANDERSEN

Paul Rubin, M.D.
Executive Vice President, R&D

XOMA | gsk | abbvie | SEPRACOR

microRNA Therapeutics Regulate Systems Biology to Modify Disease

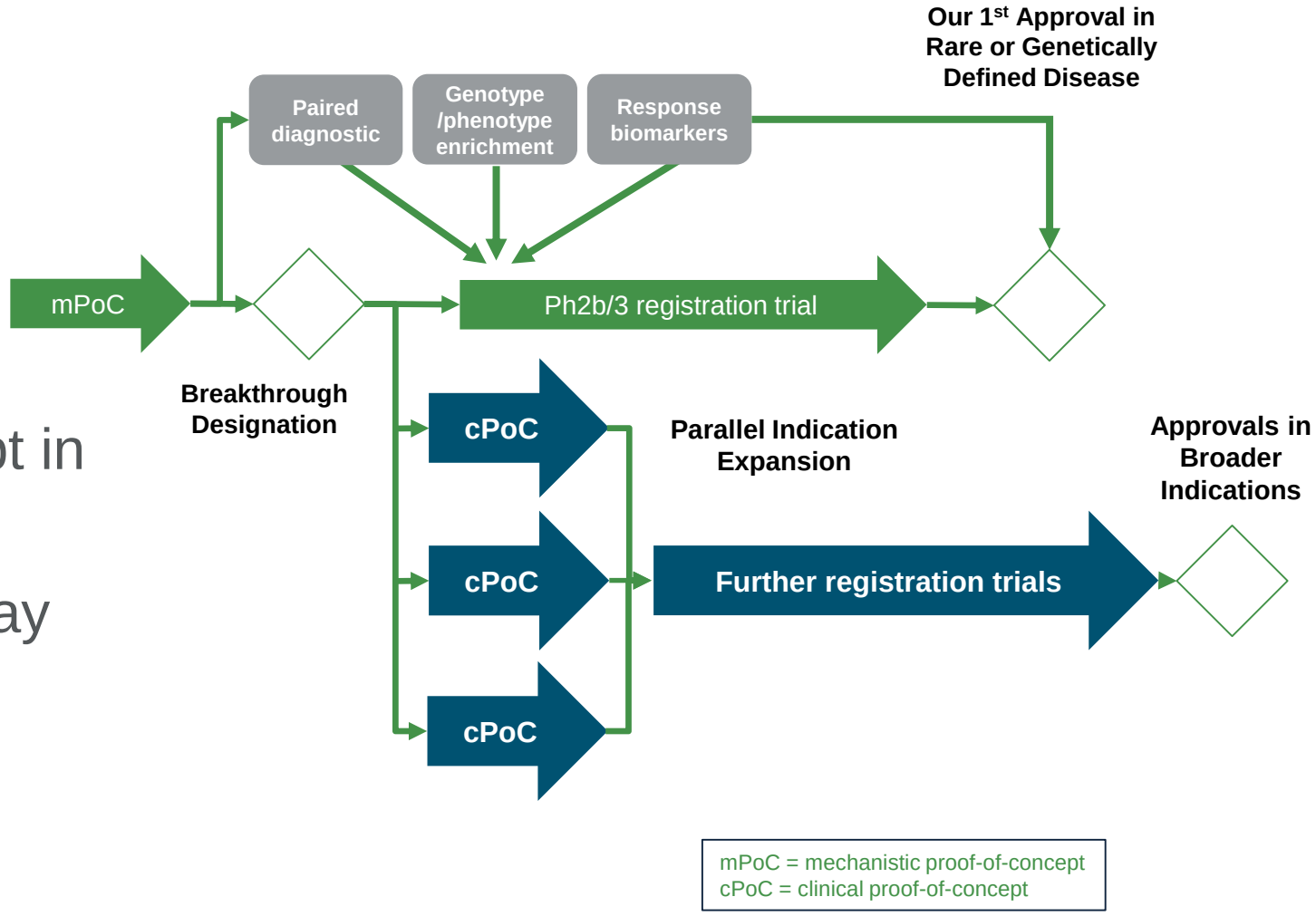


- microRNA-targeted therapy is focused on disease modification by restoring homeostasis to dysregulated processes
- microRNAs regulate complex biological systems
- microRNA-targeted therapies are intrinsically focused on disease-relevant pathways
- microRNA therapeutics particularly suited for complex, multigenic disorders

miRagen develops both microRNA inhibitors and microRNA mimics for a variety of diseases

Differentiated, Foothold Clinical Development Strategy Designed to Accelerate Timelines and Reduce Development Risk

- Biomarker-driven early clinical trials
- May reduce development risk
- May improve probability of success
- May accelerate proof-of-concept in humans
- Initial rare disease indication may allow more rapid commercialization



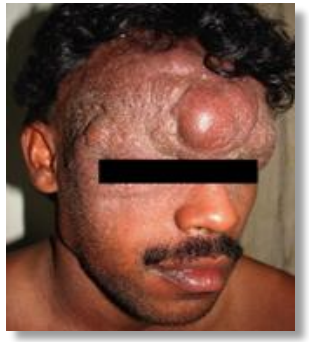
Pipeline of Product Candidates

Candidate / Target	Collaborator/ Internal	Disease Area	Pre-clinical	IND Enabling	Phase 1	Phase 2	Status / Anticipated Milestones
cobomarsen / miR-155 inhibitor	miRagen	**Blood Cancers**	Cutaneous T-cell Lymphoma Adult T-Cell Lymphoma/Leukemia Diffuse Large-B Cell Lymphoma Chronic Lymphocytic Leukemia				❑ Interim Phase 1 efficacy from longer-term duration of treatment in CTCL (1H2018) ❑ Initiation of Phase 2 trial in CTCL (2H2018) ❑ Interim Phase 1 safety and efficacy data release in expansion indication(s) (2018) ❑ Phase 2 CTCL data (2H2020)
MRG-201 / miR-29 replacement	miRagen	**Pathologic Fibrosis**	Cutaneous Fibrosis IPF[1] Ocular[2]				❑ Initiation of Phase 2a in cutaneous fibrosis (1H2018) ❑ Ocular fibrosis data release from preclinical models (1H2018) ❑ Preclinical safety and efficacy lung fibrosis data release (2018) ❑ Phase 2a cutaneous fibrosis data (2019)
MRG-107 / miR-155 inhibitor	miRagen	**Neurodegeneration**	ALS[3]				❑ Preclinical POC study underway in SOD1 ALS model
MRG-110 / miR-92 inhibitor	miRagen / SERVIER	**Ischemia**	Heart Failure Incisional Complications				❑ Initiation of 2 Phase 1 clinical trials (1H2018)

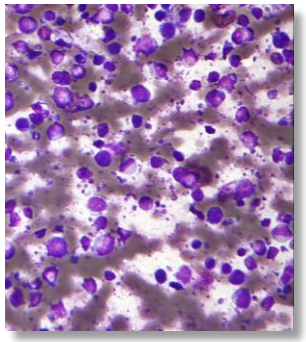
1 Idiopathic Pulmonary Fibrosis
2 Ocular Fibrosis
3 Amyotrophic Lateral Sclerosis

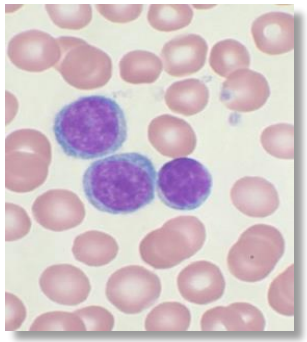
Cobomarsen (miR-155 Inhibitor) Potential Clinical Plan in Hematological Malignancies

CTCL
Mycosis Fungoides

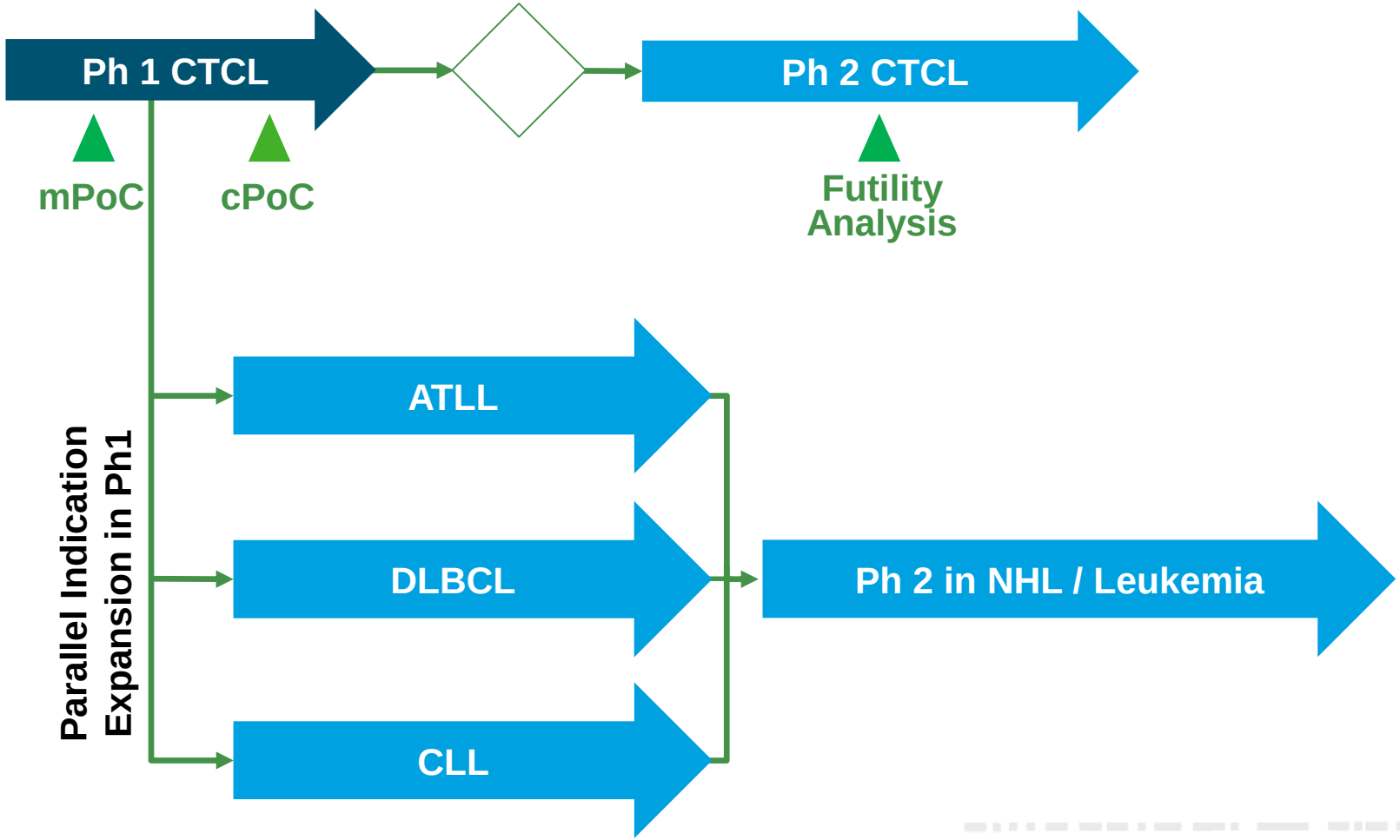


miR-155-high Non-Hodgkins Lymphoma (NHL)/Leukemia





Dose, Schedule Optimization and Response Durability in CTCL



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CTCL: A Challenging Disease in Need of Better Treatment Options

Substantial patient population

- Mycosis Fungoides prevalence of 16,000-20,000 cases in the United States
- Approximately 3,000 new diagnoses per year
- Five-year survival of approximately 90% in newly diagnosed CTCL patients

High morbidity and quality of life detriment

- Disease is disfiguring and extremely uncomfortable
- Severe itching, rash, breakdown of skin barrier
- Patients are prone to skin and blood infections, which may cause death

Low patient and physician satisfaction with existing options

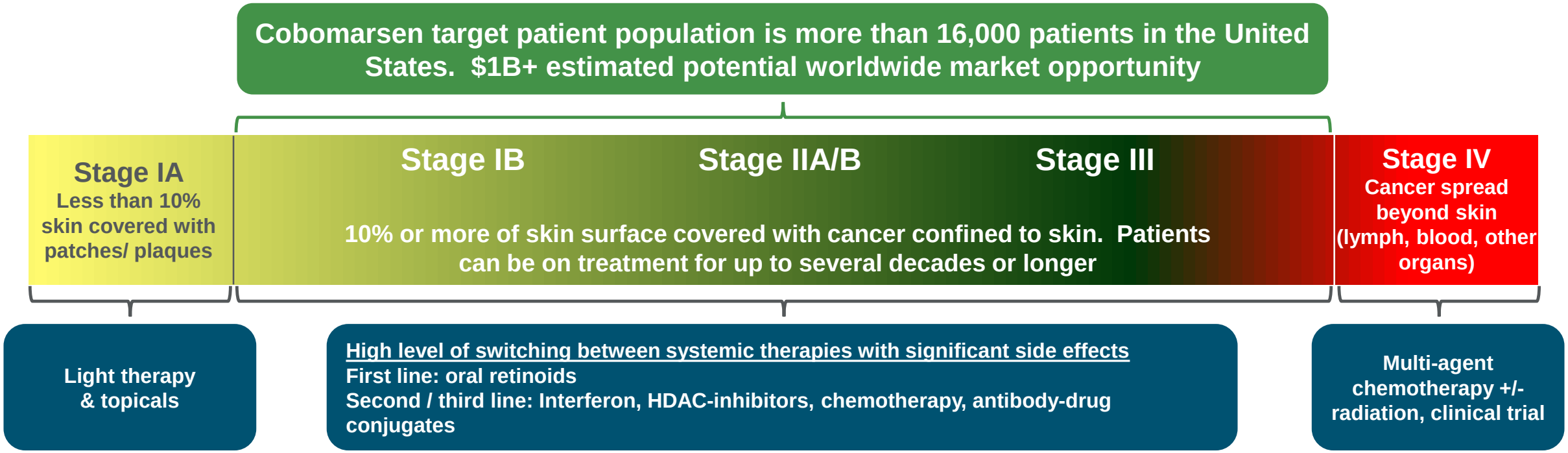
- Current treatment options have low objective response rates with limited durability of response
- Some recently approved therapies have not gained traction
- Prices for recently approved drugs range from $125,000 – $350,000/yr

Therapeutics in development target subsets of population

- Some drugs in development have shown activity in subsets of the CTCL population but with frequent side effects

Large, Unmet Market Opportunity for Cobomarsen in CTCL

"MF is a chronic, long-term challenge. Most patients, myself included, have required many different treatments over the course of time…. **A therapy that is well-tolerated and maintains its effectiveness over time is critical to individuals living with this disease."**
– Susan Thornton, CEO, Cutaneous Lymphoma Foundation, MF patient 26+ years



Our First-In-Human Phase 1 Clinical Trial of Cobomarsen in Patients with Mycosis Fungoides

- Cobomarsen is an optimized oligonucleotide inhibitor of miR-155 formulated in saline
- Objectives:

  Primary:
  - Investigate safety & tolerability of multiple intra-lesional, subcutaneous or intravenous injections

  Secondary:
  - Characterize the pharmacokinetic profile
  - Identify the recommended dose and route for a Phase 2 clinical trial
  - Evaluate the efficacy of cobomarsen in subjects with MF

  Exploratory:
  - Gene expression alterations
  - Clinical disease progression
  - Histopathology
- Patients permitted to continue CTCL therapy if on stable dose for four weeks or more prior to cobomarsen administration

First-In-Human Phase 1 Clinical Trial of Cobomarsen in Patients with Mycosis Fungoides – Trial Progression

Local Administration

- Improvement in tumors observed in all patients evaluated (n=6)
- Rapid improvement when delivered locally at high concentration (75mg intralesional injection)
- **Cobomarsen inactivated STAT, PI3K/AKT and NFkB pathways**
- Responses observed in distal tumors

Systemic Safety / Efficacy

- **90% (26/29) patients experienced clinical improvement in disease (mSWAT)**

Durability & Dose Selection

- **100% (n=8) patients receiving long term treatment at any dose level achieved greater than 4 month durable response**
- Similar efficacy and tolerability at 300mg, 600mg IV; most consistent response rate
- **75% (6/8) patients eligible for > 1 month of 300mg or 600mg IV dosing achieved ≥ 50% mSWAT reduction**

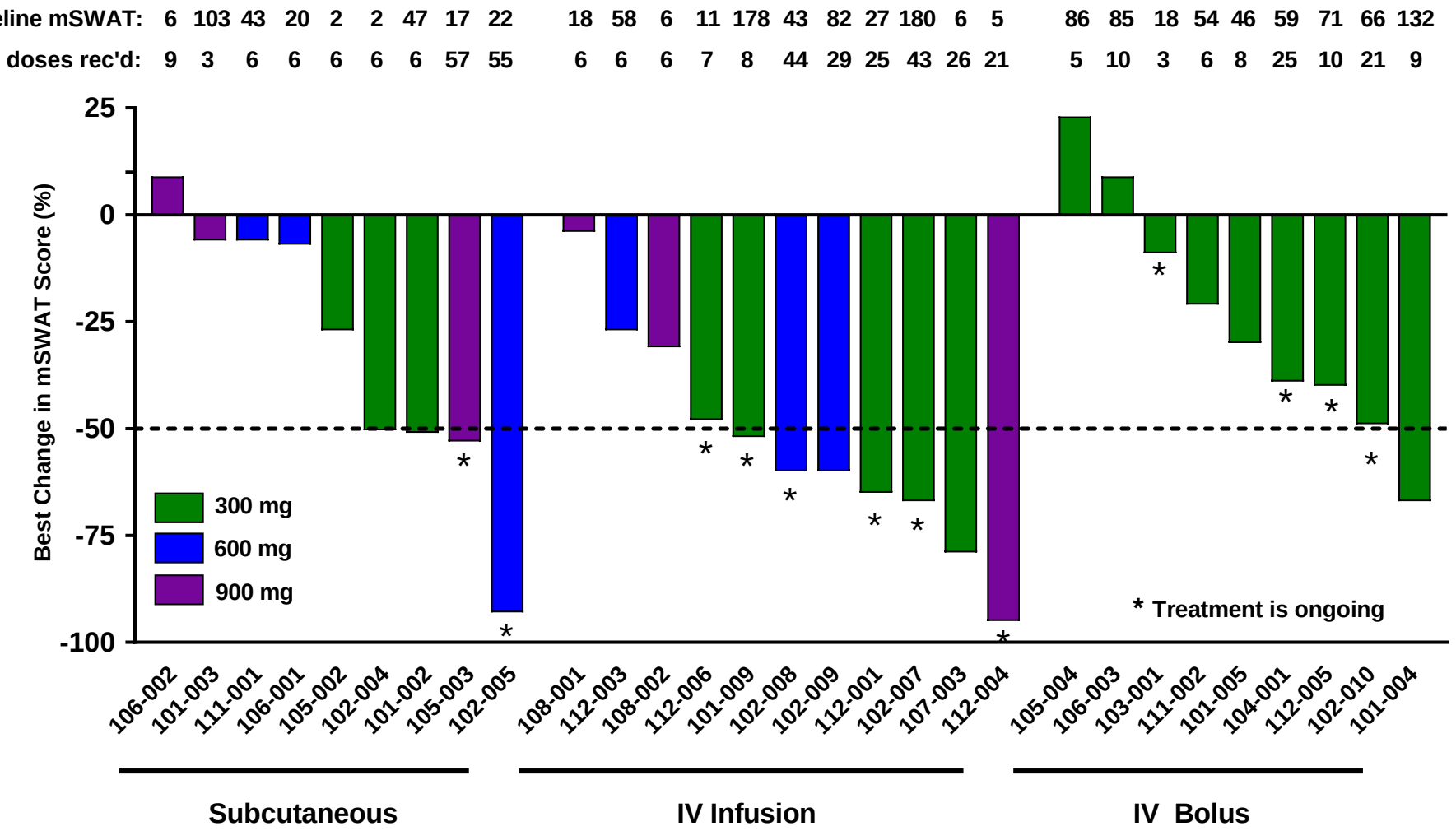
Controlled Efficacy / Durability Trial

- Controlled trial in mycosis fungoides anticipated to begin in 2H2018
- Anticipated dose: 300mg IV
- Enrollment: ~65 per group
- Proposed primary endpoint: durable response for at least 4 months
- Inclusion: Stages IB-III (mSWAT >10 with only skin involvement)
- We believe that the clinical trial could provide data that may support accelerated approval

Cobomarsen has been generally well-tolerated at all dose levels and routes of administration tested to date

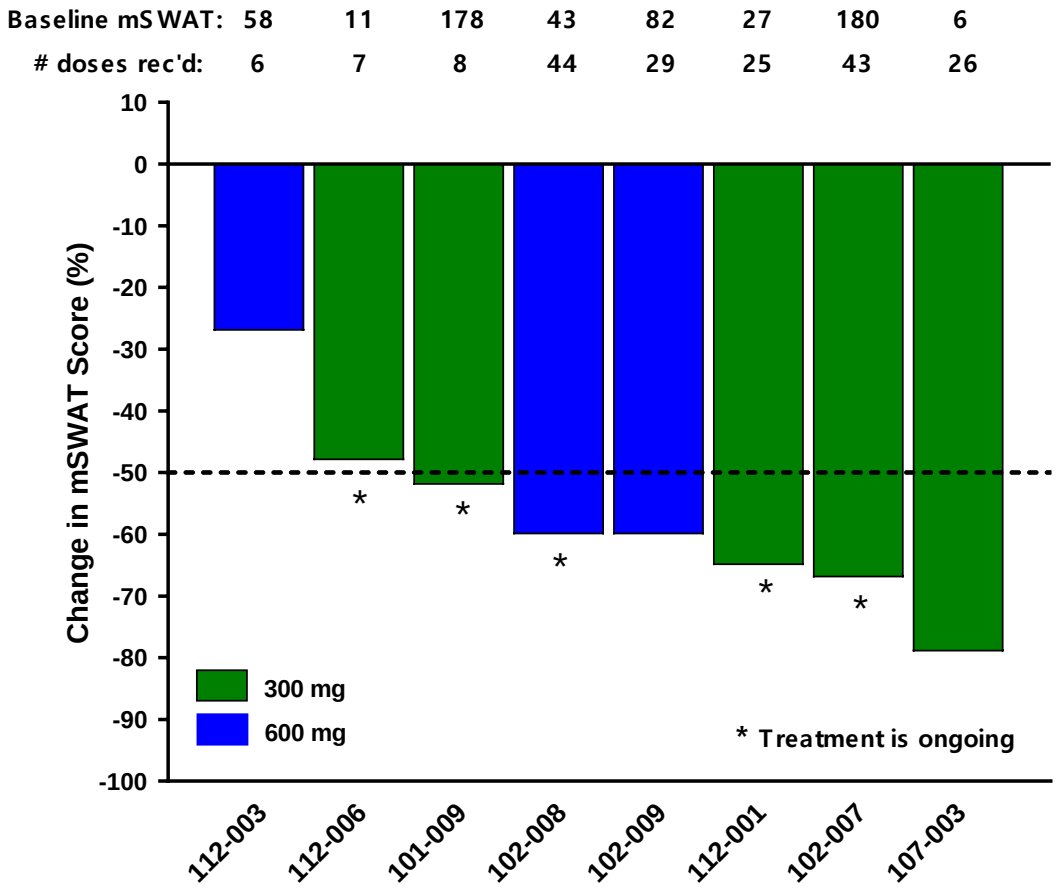
Note: Database January 25, 2018

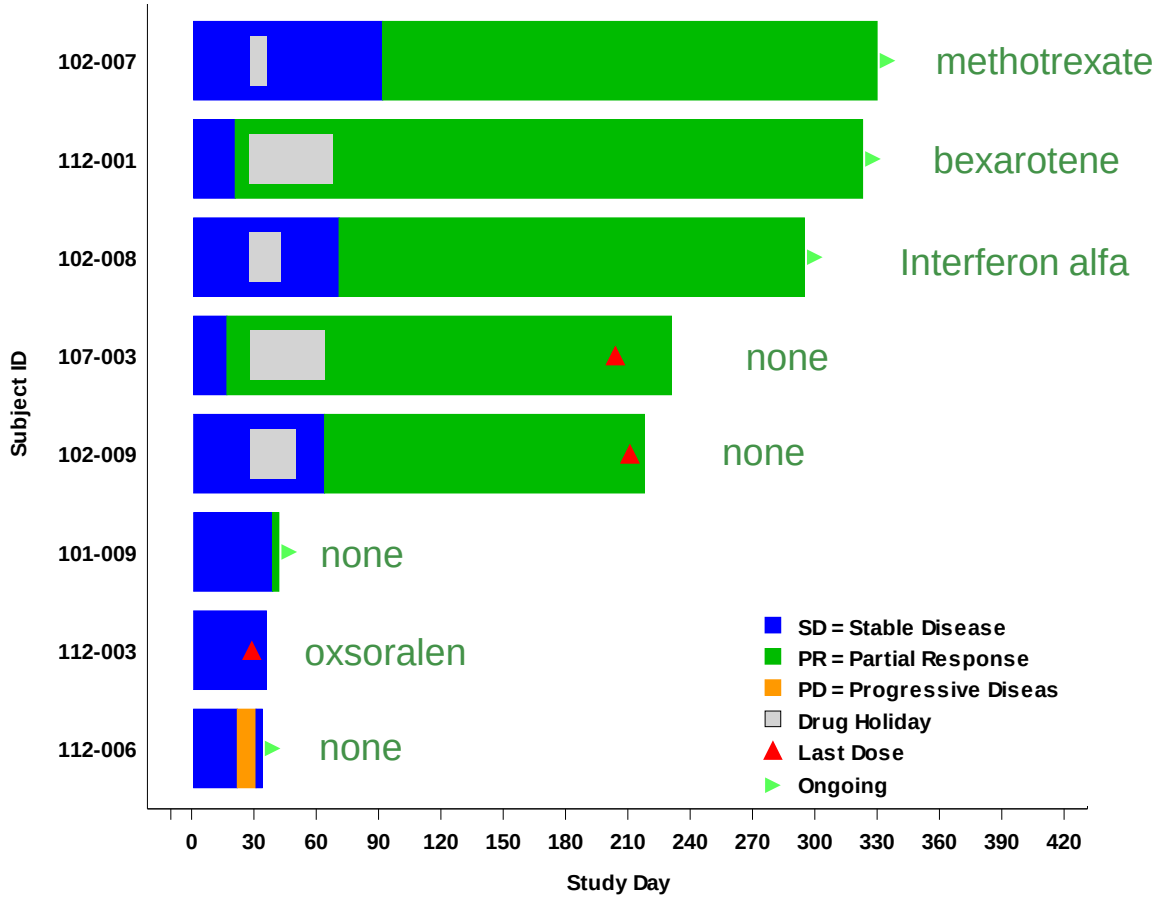
Twenty-six of Twenty-nine Subjects Treated Systemically with Cobomarsen Showed mSWAT Score Improvement



Note: Database January 25, 2018

Six of Eight (75%) Patients Eligible for More Than One Month of 300mg and 600mg IV Dosing of Cobomarsen Achieved ≥50% mSWAT Reduction





300mg Dose Selected for Phase 2 in MF; 600mg Initial Dose Selected for Phase 1 Expansion Indications

Cobomarsen Shows Favorable Tolerability

No Serious Adverse Events attributed to cobomarsen

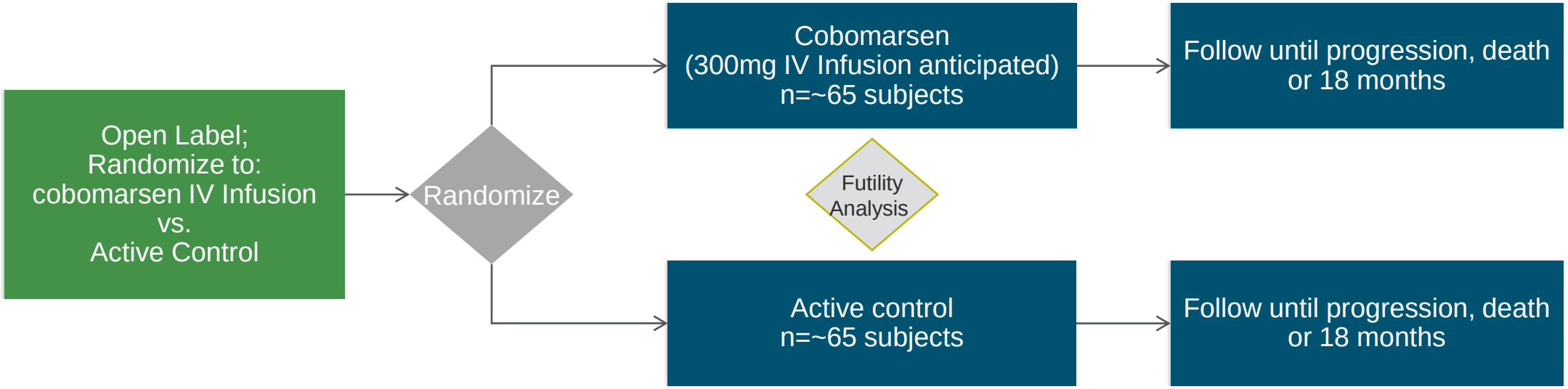
No acute inflammatory toxicities

No significant abnormalities found in liver, kidney or blood

- Cobomarsen has been safe and generally well tolerated at all doses tested
  - Multiple patients receiving more than a year of therapy (up to 39 grams cumulative dose) with no serious adverse events attributed to cobomarsen
- No significant abnormalities found in liver function, kidney function and platelet counts
- No acute inflammatory toxicities
- Novel oligonucleotide drug class
  - Elimination of “gap” reduces chemical class based toxicity
  - Short length minimizes heparin mimetic activity

Cobomarsen SOLAR Phase 2 Clinical Trial Anticipated to Initiate in 2H18

A Randomized, Parallel, Open Label, Active Control, Global Trial in Patients with Stage Ib-III Mycosis Fungoides



Primary endpoint:

- Overall Response Rate of four months (ORR4) using Global Response

Key Secondary endpoints:

- Progression-free survival
- Patient reported outcomes
  - Pain, itching

Key inclusion criteria

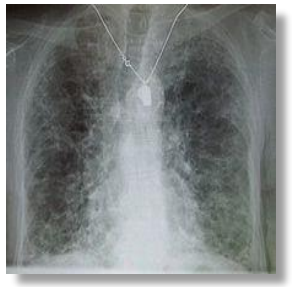
- Stage Ib-III
- Must have received at least one prior therapy for CTCL (per NCCN guidelines for generalized skin involvement)
- mSWAT score ≥ 10
- No concurrent systemic therapy

Stratification factors

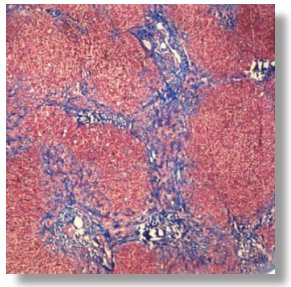
- Stage (Ib-IIa vs IIb-III)
- Prior Therapies (1-2 vs. 3 or more)

MRG-201 (miR-29 Replacement) Potential Clinical Development Plan in Fibrosis

Skin

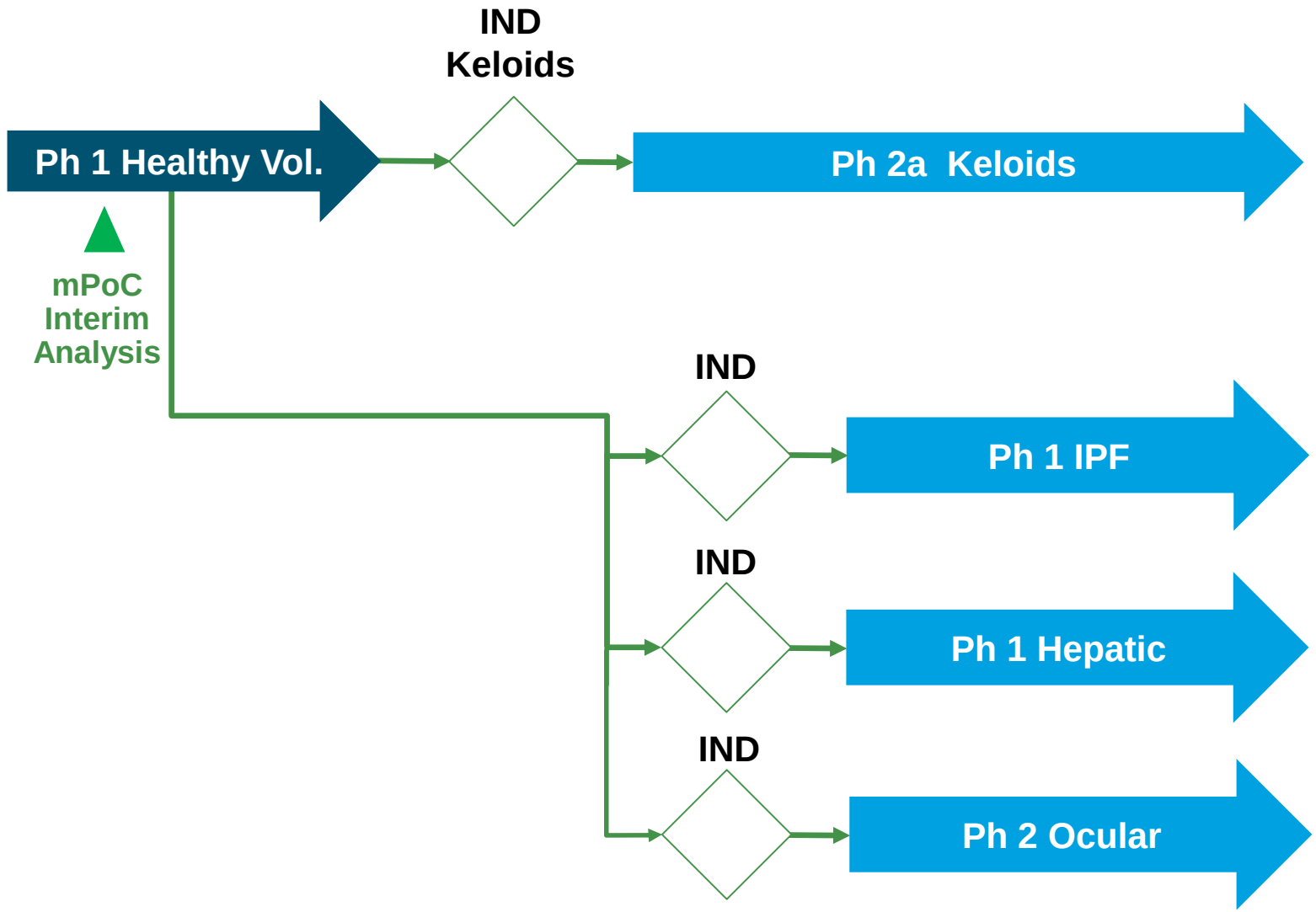


Lung



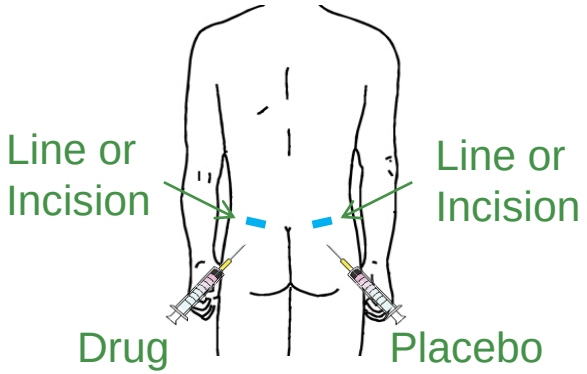
Liver

Eye



Our First-In-Human Phase 1 Clinical Trial of MRG-201 in Subjects with Induced Cutaneous Fibrosis

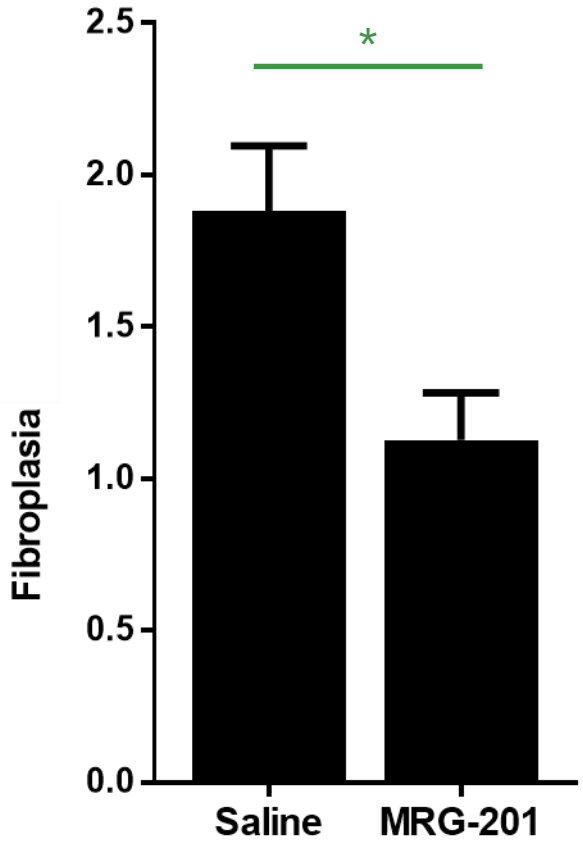
- Healthy volunteers, fibrosis induced by incisional wounding
- Four cohorts (3-10 subjects per cohort):
  - A – establish PD marker kinetics in skin incision ✓
  - B – single ascending dose in intact skin ✓
  - C – single ascending dose around skin incision ✓
  - D – multiple ascending doses around skin incision ✓



- MRG-201 at doses of 0.5-14mg in all cohorts was generally well tolerated
- MRG-201 treatment inhibits expression of fibrogenesis biomarkers in humans

Blinded Histology Analysis Showed Statistically Significant Reduction of Fibroplasia Without Affecting Wound Healing in Human Phase 1 Clinical Trial

- MRG-201 treatment appears to inhibit the expression of dynamic and mechanistic biomarkers of fibrogenesis in humans
- This appears to result in a significant reduction in fibroplasia, a marker of scar tissue deposition
- Normal regranulation and healing of the wounds observed with treatment
- Suggests potential for broad utility in scar reduction if approved



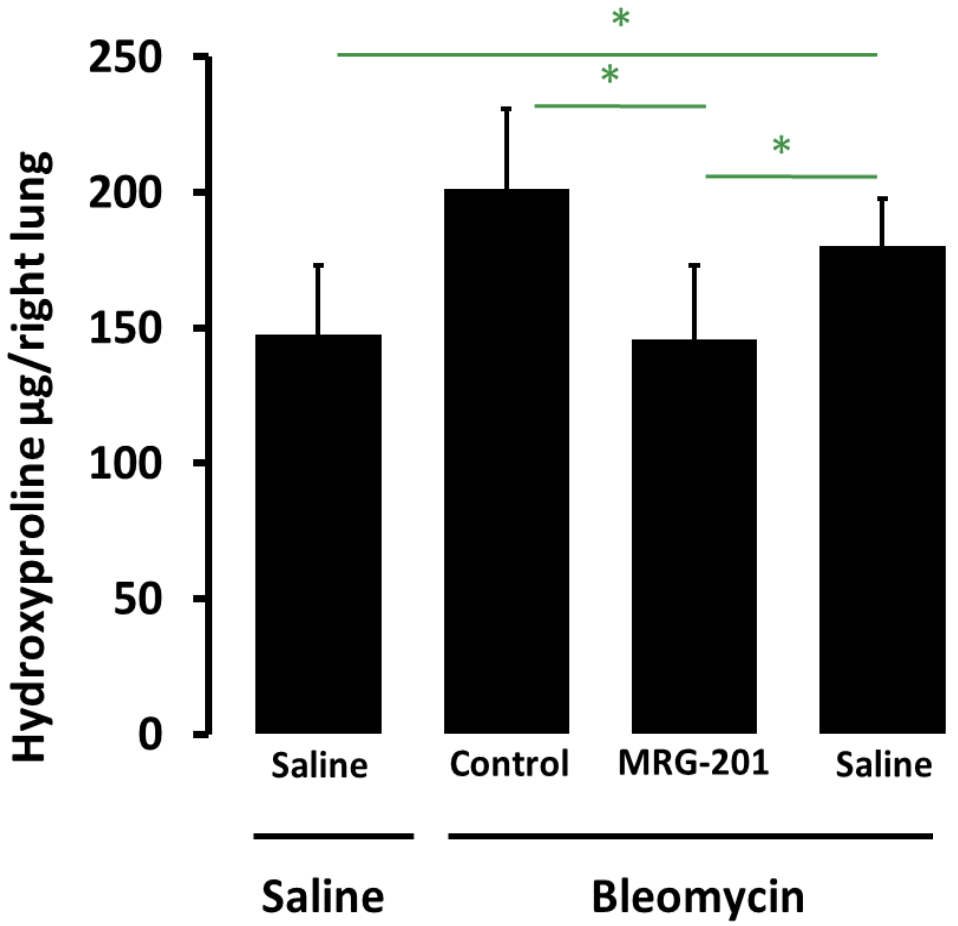
16 subjects
(Additional 3 subjects did not have histology assessment performed)

Hematoxylin and Eosin stain & assessment by a blinded pathologist

*Statistically significant (p=0.0086)

Nebulized MRG-201 Attenuates Fibrosis Induced by Bleomycin in Preclinical Model of Pulmonary Fibrosis

- MRG-201 appears stable to nebulization with intact chemical structure
- MRG-201 accumulates in lung tissue after inhalation
- Fibrogenesis biomarkers appear inhibited by MRG-201 treatment
- Reversal of fibrotic tissue deposition in the lung observed with treatment



*Statistically significant ($p<0.05$)

Note: MRG-201 or control dosing started 10 days after bleomycin administration – administered daily for 7 days. Study performed at Yale.

Recent Events and Anticipated Milestones

Program	2017	2018
Blood Cancers (cobomarsen)	✓ Interim Phase 1 clinical trial CTCL data presentation at ASCO ✓ Phase 1 clinical trial expansion to include ATLL, DLBCL, CLL ✓ Interim Phase 1 clinical trial CTCL data presentation at ASH	❑ Interim Phase 1 efficacy from longer-term duration of treatment in CTCL (1H) ❑ Initiation of Phase 2 clinical trial in CTCL (2H) ❑ Phase 1 clinical trial data release in expansion indication(s)
Pathologic Fibrosis (MRG-201)	✓ Last patient dosed in Phase 1 clinical trial dermatologic fibrosis trial ✓ Preclinical inhalation feasibility study results presentation at scientific conference ✓ Phase 1 results presentation at scientific conference	❑ Initiation of Phase 2a clinical trial in cutaneous fibrosis (1H) ❑ Preclinical safety and efficacy lung fibrosis data release ❑ Ocular fibrosis data release from preclinical models (1H)
Increased Neovascularization (MRG-110)	✓ Completion of IND/CTA enabling studies	❑ Initiation of two Phase 1 clinical trials (1H)

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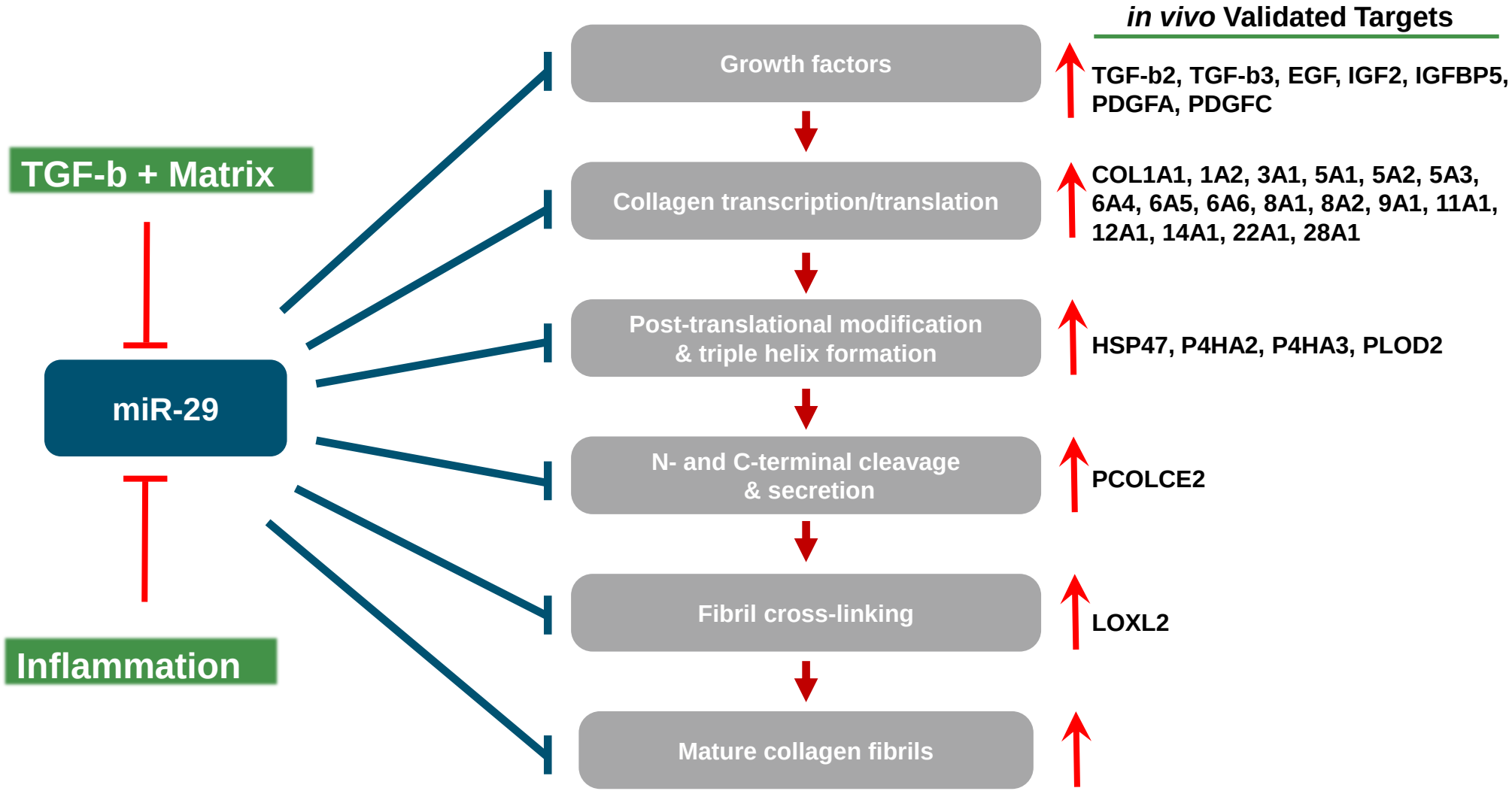
March 2018

miR-29 is a Regulator of Biological Pathways Implicated in Fibrosis



Regulating Systems Biology to Modify Disease

miR-155 is an OncomiR and a Pro-inflammatory microRNA