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 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, 2018 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 RNA-targeted drug discovery and development with next generation nucleic acid therapeutics platform
- Cobomarsen in hematological malignancies
  - Human Phase 1 clinical proof-of-concept achieved in Cutaneous T-Cell Lymphoma (CTCL)
  - Controlled Phase 2 clinical trial in CTCL recently initiated, data could allow for accelerated approval
  - Promising clinical results obtained in treatment of Adult T-cell Leukemia/Lymphoma (ATLL)
- Remlarsen in pathological fibrosis
  - Mechanistic proof-of-concept in dermal scarring obtained from Phase 1 clinical trial
  - Phase 2 clinical trial in pathological cutaneous fibrosis should read out in the second half of 2019
  - Preclinical data in ocular fibrosis provides attractive next clinical indication
- MRG-110 in cardiovascular disease
  - Two Phase 1 clinical trials for systemic and local administration should read out in 2019
  - Development path moving forward in central and peripheral indications will be defined in 2019
  - Development funded by Servier; miRagen retains commercial rights in the United States and Japan
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
## Pipeline of Product Candidates

<table>
<thead>
<tr>
<th>Candidate / Target</th>
<th>Collaborator/Internal</th>
<th>Disease Area</th>
<th>Pre-clinical</th>
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✓ Final CTCL Phase 1 data (4Q2018)  
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✓ Additional Phase 1 safety and efficacy data in ATLL (1H2019)  
❑ Phase 2 CTCL data (2H2020) |
| remlarsen / miR-29 replacement | miragen | Pathologic Fibrosis | Cutaneous Fibrosis | | Ocular¹ | ✓ | Ocular fibrosis data release from preclinical models  
✓ Initiation of Phase 2 in cutaneous fibrosis  
✓ Preclinical ocular fibrosis data (2Q2019)  
✓ Preclinical lung fibrosis data (2019)  
❑ Phase 2 cutaneous fibrosis data (2H2019) |
| MRG-110 / miR-92 inhibitor | miragen/Server | Ischemia | Heart Failure | | Incisional Complications | ✓ | Phase 1 clinical trial with systemic dosing initiated  
✓ Initiation of Phase 1 clinical trial with local administration  
❑ Phase 1 systemic data 2019  
❑ Phase 1 local administration data 2019 |

1 Ocular Fibrosis  
2 Idiopathic Pulmonary Fibrosis
Overexpression of OncomiR-155 Has Been Associated With Poor Outcomes in a Wide Range of Hematological Malignancies and Solid Tumors

- Cobomarsen is an inhibitor of miR-155 that has been shown to inactivate multiple disease-relevant pathways in human clinical trials
- This targeted approach allows for specific induction of pro-apoptotic and anti-proliferative pathways in cancer cells while not affecting normal cells
- By affecting multiple signaling pathways in cancer, cobomarsen may provide long term benefit with few potential resistance mechanisms
- miRagen is pursuing a step-wise “foothold” clinical evaluation program in multiple hematological malignancies where miR-155 is overexpressed
Cobomarsen Clinical Plan in Hematological Malignancies

CTCL
Mycosis Fungoides

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

Dose, Schedule Optimization and Response Durability in CTCL

Ph 1 CTCL

mPoC

cPoC

Ph 2 SOLAR Trial

Parallel Indication Expansion in Ph1

ATLL

DLBCL

CLL

Ph 2 in NHL / Leukemia

Futility Analysis
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
Cobomarsen Phase 1 Trial in MF Final Data Highlights

- 92% of the MF subjects in the systemic administration cohorts had improvement in tumor burden as assessed by mSWAT score independent of concomitant therapies
- 52% of patients receiving more than 6 doses achieved a partial response (at least a 50% reduction) in mSWAT score
- For the evaluable patients achieving a PR (n=12), the mean duration of response was 276 days at the time of the January 9, 2019 data cutoff
- 63% of subjects treated with cobomarsen administered as a 300 mg IV-infusion achieved a PR and 50% maintained the response for greater than four months (ORR4)
- Cobomarsen was generally well-tolerated at all doses tested
Thirty-three of Thirty-six Subjects (92%) Treated Systemically with Cobomarsen Have Shown mSWAT Score Improvement

- mSWAT score represents best score achieved while on study for 36 patients who had evaluable mSWAT scores as of the data cutoff
- Duration of response (days) for each evaluable patient achieving a 50% reduction in mSWAT score is shown in individual bar
- NE = Not Evaluable; patients not allowed to continue on trial as per protocol or lost to follow up

Note: Database January 9, 2019
Five of Eight (63%) Subjects Treated with Cobomarsen Administered as a 300 mg IV-infusion Achieved a PR
Cobomarsen has been generally well tolerated at all doses tested

- 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

No Serious Adverse Events attributed to cobomarsen

No acute inflammatory toxicities

No significant abnormalities found in liver, kidney or blood
Cobomarsen SOLAR Phase 2 Clinical Trial Initiated in 4Q18
A Randomized, Parallel, Open Label, Active Control, Global Trial in Patients with Stage Ib-III Mycosis Fungoides

Open Label; Randomize to:
cobomarsen IV Infusion
vs. vorinostat

Randomize

Cobomarsen
(300mg IV Infusion anticipated)
n=~65 subjects

Follow until progression

Futility
Analysis

vorinostat
n=~65 subjects

Follow until progression

Primary Endpoint
• Overall Response Rate of four months (ORR4) using Global Response

Key Secondary Endpoint
• Progression-free survival

Additional Secondary Endpoint
• Patient reported outcomes
  • Skindex29, pruritus, pain

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
MAVORIC: Open-label, Randomized Study of Mogamulizumab vs Vorinostat in CTCL

Inclusion:
- Stage IB – IVB, MF or SS (B2)
- At least one prior course of systemic therapy

Exclusion:
- Patients with large cell transformation

1:1 Randomization

Mogamulizumab
1.0 mg/kg i.v.
Weekly for first 28-day cycle; days 1 and 15 of subsequent cycles

Vorinostat
400 mg PO daily

One-way crossover after PD or intolerable toxicity

<table>
<thead>
<tr>
<th></th>
<th>Mogamulizumab</th>
<th>Vorinostat</th>
</tr>
</thead>
<tbody>
<tr>
<td>Median PFS (Months)</td>
<td>7.7</td>
<td>3.1</td>
</tr>
<tr>
<td>ORR (%)</td>
<td>28</td>
<td>4.8</td>
</tr>
<tr>
<td>&gt; Grade 3 TEAEs (%)</td>
<td>4.3</td>
<td>5.9</td>
</tr>
<tr>
<td>Common TEAEs (&gt;20%)</td>
<td>infusion Rx, diarrhea, fatigue</td>
<td>diarrhea, nausea, fatigue, thrombocytopenia, etc.</td>
</tr>
</tbody>
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ATLL: Adult T-cell Leukemia / Lymphoma

- Adult T-cell leukemia/lymphoma (ATLL) is a peripheral T-cell neoplasm caused by human T-lymphotrophic virus type 1 (HTLV-1)
- HTLV-1 is correlated with the overexpression of miR-155 during its lifecycle
- Malignant transformation leading to ATLL occurs in up to 7% of HTLV-1–infected individuals
- The aggressive form of the disease is highly morbid, with mean survival times of 4-10 months after diagnosis even with current best standard of care

Arch Pathol Lab Med 2014;138-282; Blood Advances, 2018 (march27); 2 (6)
Cobomarsen Treatment Results in Durable Clinical Stabilization of Disease after Chemotherapy in Five Patients for up to 16 Months

- Nine patients with ATLL have been treated with cobomarsen
- Four of 5 patients remained stable for up to 16 months and continue on the trial as of the data cut off
- Three subjects, one relapsing lymphomatous and two relapsing with significant skin involvement, were treated for less than one month and withdrew from study
- Patients on cobomarsen directly after chemotherapy have shown a restoration and maintenance of their red blood cell, white blood cell and platelet counts
  - No evidence of opportunistic infections
- Cobomarsen was generally well tolerated in all patients
Remlarsen (miR-29 Replacement) Potential Clinical Development Plan in Fibrosis

1 Next generation miR-29 replacement
Blinded Histology Analysis Showed Statistically Significant Reduction of Fibroplasia Without Affecting Wound Healing in Human Phase 1 Clinical Trial

- Remlarsen 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
- Justifies further exploration for broad applications in scar reduction

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)
Remlarsen Cutaneous Fibrosis Phase 2 Clinical Trial Design

- Only patients with history of frequent keloid formation after trauma will be entered (12 pts/cohort up to five cohorts)
- Double blind randomized trial with patients serving as their own control

- Subjects undergo excisional wounds via two 6-mm biopsies bilaterally in the upper back/shoulder
  - One wound will be treated with remlarsen and the other will be treated with placebo given intradermally
- Subjects observed every 4 weeks for 1 year to assess for keloid formation
Remlarsen as an Anti-Fibrotic in the Eye

- Successful biodistribution to target cells
  - Retina with intravitreal injection
  - Cornea with topical administration
- Compelling biomarker and antifibrotic activity

![Image showing successful biodistribution and antifibrotic activity in the eye](image-url)
MRG-110 Treatment Leads to Increased Collateral Growth and Improved Function in Porcine Model of Chronic Myocardial Ischemia
# Summary of Recent Events and Anticipated Milestones

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1 Ocular Fibrosis  
2 Idiopathic Pulmonary Fibrosis
miR-29 is a Regulator of Biological Pathways Implicated in Fibrosis

**TGF-b + Matrix**

*miR-29*

**Inflammation**

**Growth factors**

**Collagen transcription/translation**

**Post-translational modification & triple helix formation**

**N- and C-terminal cleavage & secretion**

**Fibril cross-linking**

**Mature collagen fibrils**

*in vivo* Validated Targets

- TGF-b2, TGF-b3, EGF, IGFBP5, PDGFA, PDGFC
- COL1A1, 1A2, 3A1, 5A1, 5A2, 5A3, 6A4, 6A5, 6A6, 8A1, 8A2, 9A1, 11A1, 12A1, 14A1, 22A1, 28A1
- HSP47, P4HA2, P4HA3, PLOD2
- PCOLCE2
- LOXL2

miR-29 targets include:

- **Growth factors**
- **Collagen transcription/translation**
- **Post-translational modification & triple helix formation**
- **N- and C-terminal cleavage & secretion**
- **Fibril cross-linking**
- **Mature collagen fibrils**

miR-29 regulates biological pathways implicated in fibrosis.
Regulating Systems Biology to Modify Disease

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

↑ miR-155

- CEBPb: Inflammation M1→M2
- SOCS: iNOS, Cytokines, T cell activation
- SHIP-1: PI3K/AKT/MAPK, Proliferation, Myeloid expansion
- Jarid2: Leukemic transformation
- PU.1: Myeloid differentiation
- Wee1: DNA repair

Inflammation / Immunity

- IL-6, TNFa
- IL-10, IL-12p40
- B cell and DC maturation

Cancer