First Quarter 2021 Financial Results Earnings Call

Stealth BIOTHERAPEUTICS

Leading Mitochondrial Medicine

May 18, 2021

First Quarter 2021 Earnings Call

Forward Looking Statements

- **HENRY HESS,** *Chief Legal Counsel*

Introduction and Business Highlights

- **REENIE MCCARTHY,** *Chief Executive Officer*

Update on Pipeline Programs

- **JIM CARR,** *Chief Clinical Development Officer*
- **BRIAN BLAKEY,** *Chief Business Officer*

Financial Results Q1 2021

- **ROB WEISKOPF,** *Chief Financial Officer*

Questions & Answers

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Forward-looking Statements

This presentation and various remarks we make during this presentation contain forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Such forward-looking statements include those regarding our plans, strategies and expectations for our preclinical and clinical advancement of our drug development programs, including our ongoing clinical trials of elamipretide and planned clinical trial of SBT-272; our plans for the potential submission of an NDA; our expectations regarding regulatory interactions, including our belief that the existing data and the data from the withdrawal protocol may provide sufficient evidence to support NDA review; the potential benefits of our product candidates; our key milestones for 2021 and 2022; our plans regarding future data presentations; and our financial guidance regarding the period in which we will have capital available to fund our operations. Statements that are not historical facts, including statements about our beliefs, plans and expectations, are forward-looking statements. The words "anticipate," "expect," "hope," "plan," "potential," "possible," "will," "believe," "estimate," "intend," "may," "predict," "project," "would" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. We may not actually achieve the plans, intentions or expectations disclosed in these forward-looking statements, and you should not place undue reliance on these forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements as a result of known and unknown risks, uncertainties and other important factors, including: our ability to obtain additional funding and to continue as a going concern; the impact of the COVID-19 pandemic; the ability to successfully demonstrate the efficacy and safety of our product candidates and future product candidates; the preclinical and clinical results for our product candidates, which may not support further development and marketing approval; the potential advantages of our product candidates; the content and timing of decisions made by the FDA, the EMA or other regulatory authorities, investigational review boards at clinical trial sites and publication review bodies, which may affect the initiation, timing and progress of preclinical studies and clinical trials of our product candidates; our ability to obtain and maintain requisite regulatory approvals and to enroll patients in our planned clinical trials; unplanned cash requirements and expenditures; competitive factors; our ability to obtain, maintain and enforce patent and other intellectual property protection for any product candidates we are developing; and general economic and market conditions. These and other risks are described in greater detail under the caption "Risk Factors" included in our most recent Annual Report on Form 20-F filed with the Securities and Exchange Commission ("SEC"), as well as in any future filings with the SEC. Forward-looking statements represent management's current expectations and are inherently uncertain. Except as required by law, we do not undertake any obligation to update forward-looking statements made by us to reflect subsequent events or circumstances.

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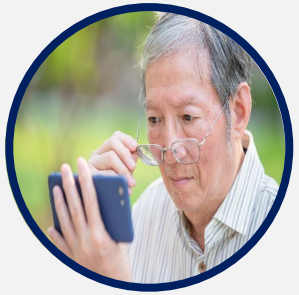
STRATEGY

CARDIOLOGY PLATFORM
Reverse remodeling the failing heart



OPHTHALMOLOGY PLATFORM
Improving vision in blinding diseases

NEUROLOGY PLATFORM
Evidence of peripheral improvements; early signs of neuronal protection

2021 KEY MILESTONES

ENROLL BARTH PHASE 3 RANDOMIZED WITHDRAWAL TRIAL *to support 2022 NDA submission, progress European regulatory initiatives and progress initiation of up to 3 additional indications*

REPORT PHASE 2 GA RESULTS IN EARLY 2022 *with Phase 2 demographics and additional Phase 1 data during 2021 to support potential to reclaim visual function; IVT feasibility ongoing*

INITIATE PHASE 3 TRIAL IN nPMD *whilst progressing next-generation clinical stage compound SBT-272 to Phase 2 readiness*

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CARDIOLOGY PLATFORM

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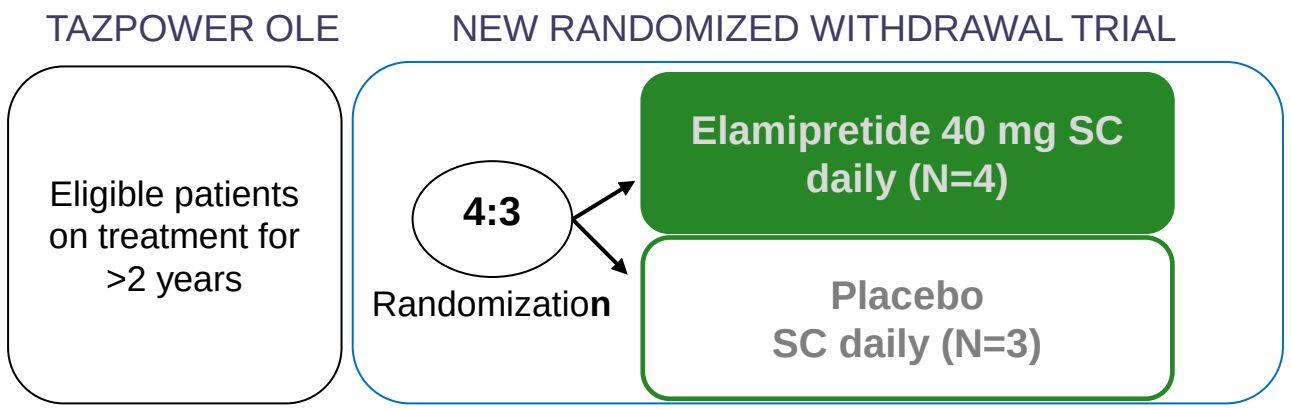
Barth: Proposed Randomized Withdrawal Trial Design



proposed p ≤ 0.10

- Primary Objective
  - To evaluate the maintenance of efficacy of single daily subcutaneous (SC) doses of 40 mg elamipretide in subjects receiving chronic treatment, and to assess the impact of withdrawal of treatment as measured by ***time to treatment failure***
- Secondary Objective
  - To evaluate the maintenance of efficacy of single daily subcutaneous (SC) doses of 40 mg elamipretide in subjects receiving chronic treatment, and to assess the impact of withdrawal of treatment as measured by changes in functional / cardiac / PRO assessments

Treatment failure

- *15% decline in 6MWT*
- *10% decline in stroke volume on cardiac MRI*
- *Hospitalization or ER visit due to disease progression (excluding infections) in opinion of PI*
- *Subject withdrawal of consent from the trial due to disease progression*
- *Patient report of moderately or very much worsening of disease on PGI–C scale*
- *Addition of or increase in con-meds to control HF or implantable defibrillator + CRT*

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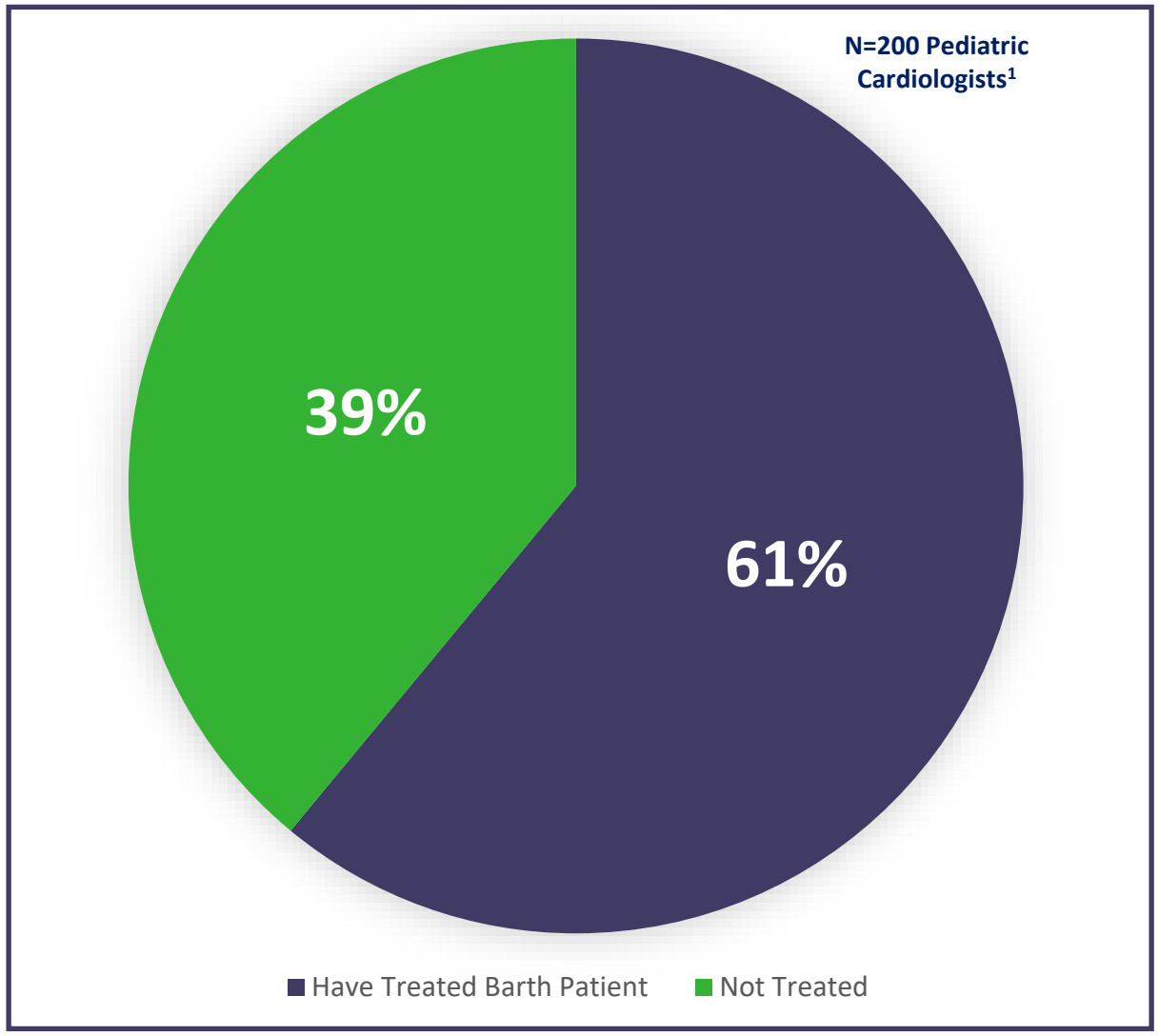
Broadening Awareness of Unmet Need in Barth



Publications and Presentations Support the Potential of Elamipretide as the First Treatment for Barth

nature genetics

A phase 2/3 randomized clinical trial followed by an open-label extension to evaluate the effectiveness of elamipretide in Barth syndrome, a genetic disorder of mitochondrial cardiolipin metabolism

ASHG American Society of Human Genetics

Elamipretide Significantly Improves Disease Symptomatology versus Natural History Controls in Barth Syndrome

AMERICAN COLLEGE of CARDIOLOGY

Elamipretide Improves Functional Assessments when Compared to the Natural History Progression of Cardiomyopathy-related Disease Symptomology in Patients with Barth Syndrome

[1] Bruno and Ridgway Research Associated, April 2021, Survey of 200 Pediatric Cardiologists

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OPHTHALMOLOGY PLATFORM

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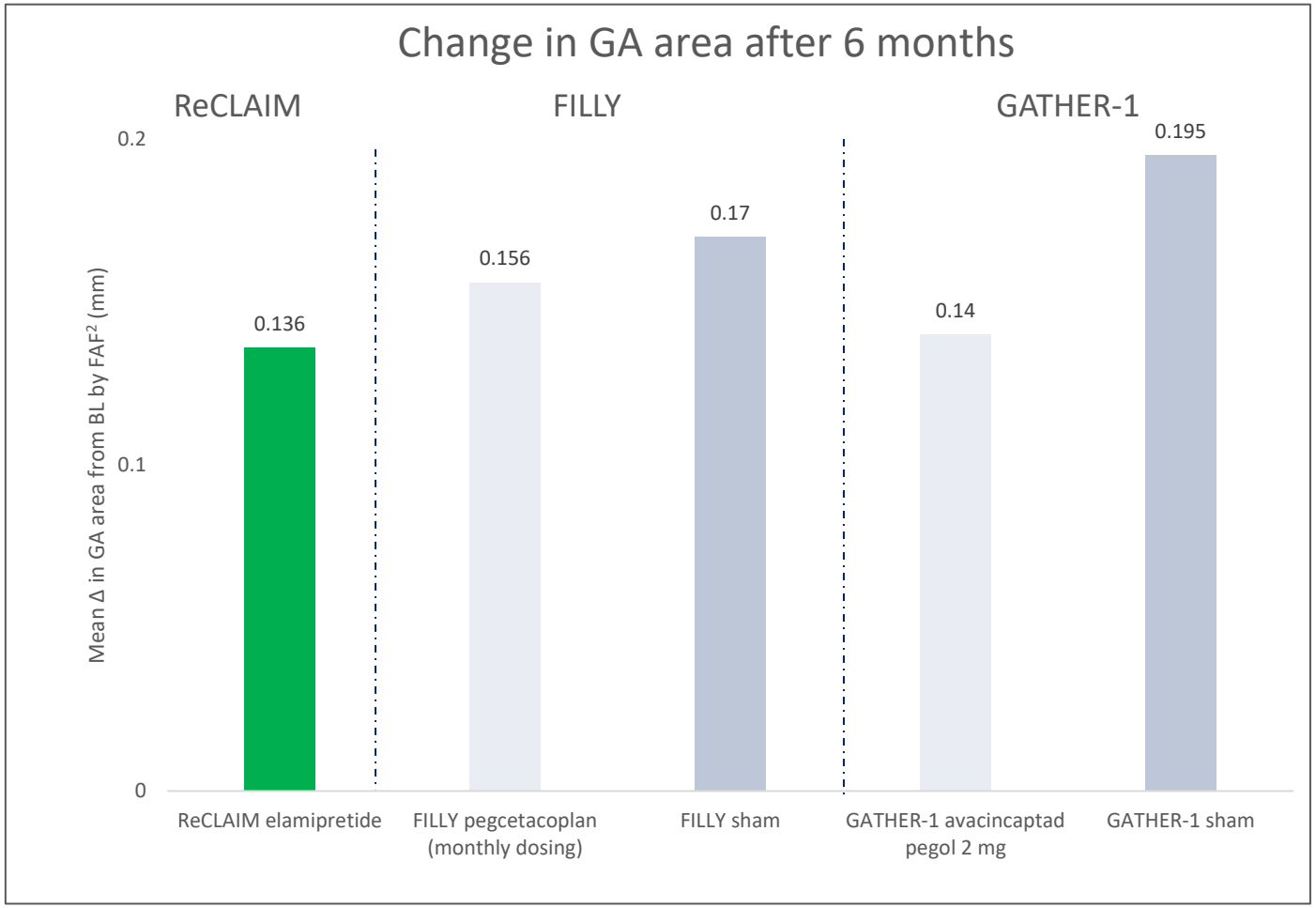
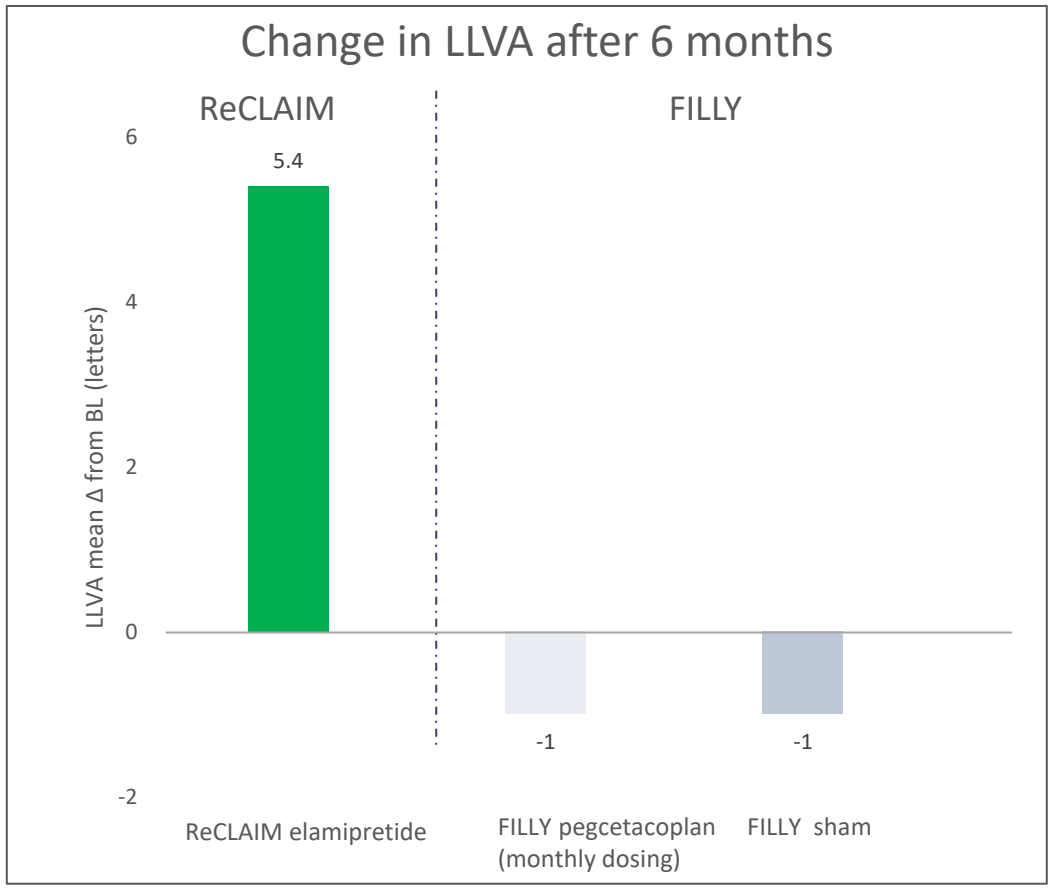
Dry AMD: ReCLAIM reduced GA growth, improved vision

Reduced GA growth and improved vision at 6 months relative to other agents in development[1]





[1]Liao et al., Ophthalmology 2020; Jaffe et al., Ophthalmology 2020, with 6-month LLVA extrapolated from graphic representation; FILLY and Gather-1 patient populations differ from ReCLAIM; FAF²=fundus autofluorescence, square root; LLVA=low light visual acuity; Δ=change; BL= baseline

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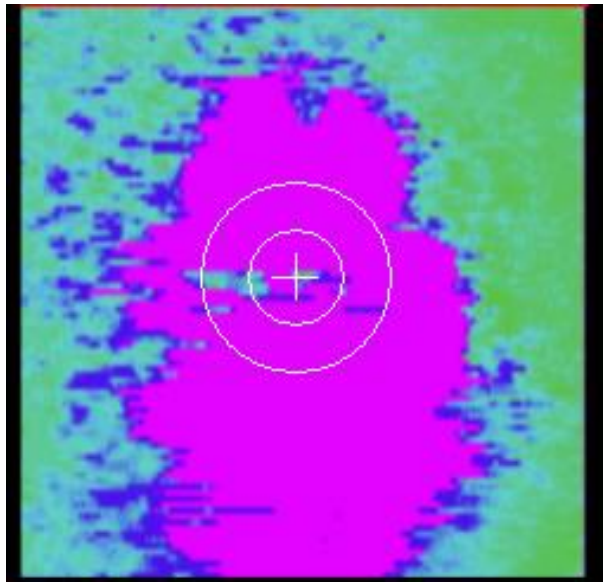
ARVO data: improved vision correlates with ellipsoid zone health

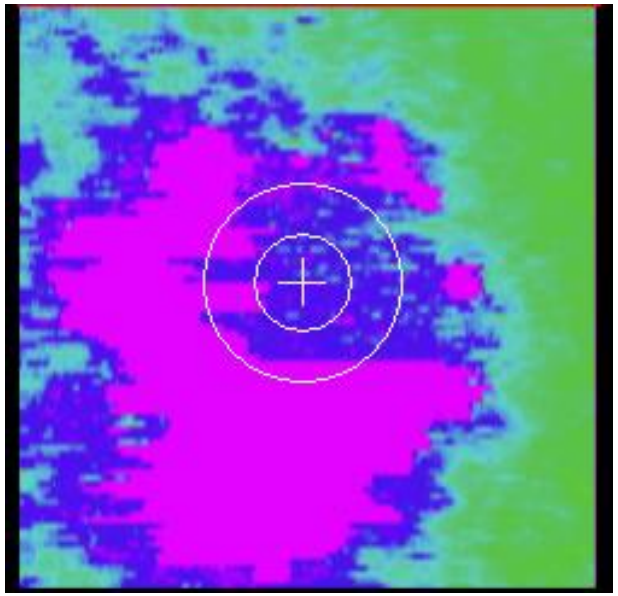
- The ellipsoid zone (EZ) is an area of the retina comprised mostly of mitochondria which supports photoreceptor function and is known to be attenuated in dry AMD
- ~50% of GA patients gained ≥5 letters in LLVA; with response correlated with baseline macular percentage of total EZ attenuation (r = -0.72; *P* = 0.002)

EZ-zone mapping from illustrative GA patients, w/ indicating EZ attenuation and indicating healthy EZ

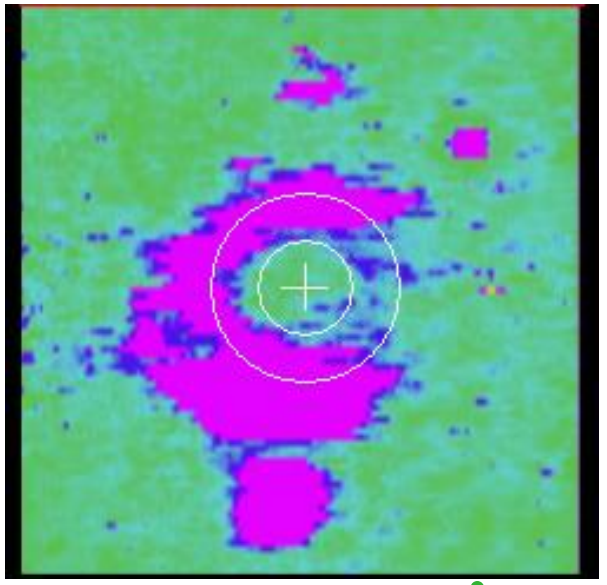
2-letter gain



4-letter gain



18-letter gain



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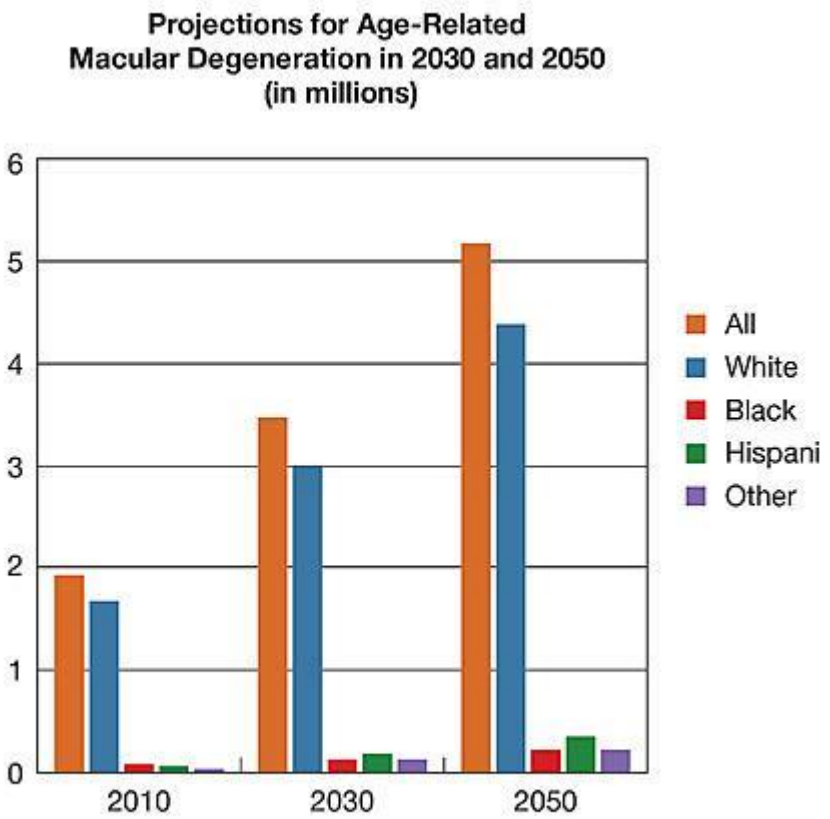
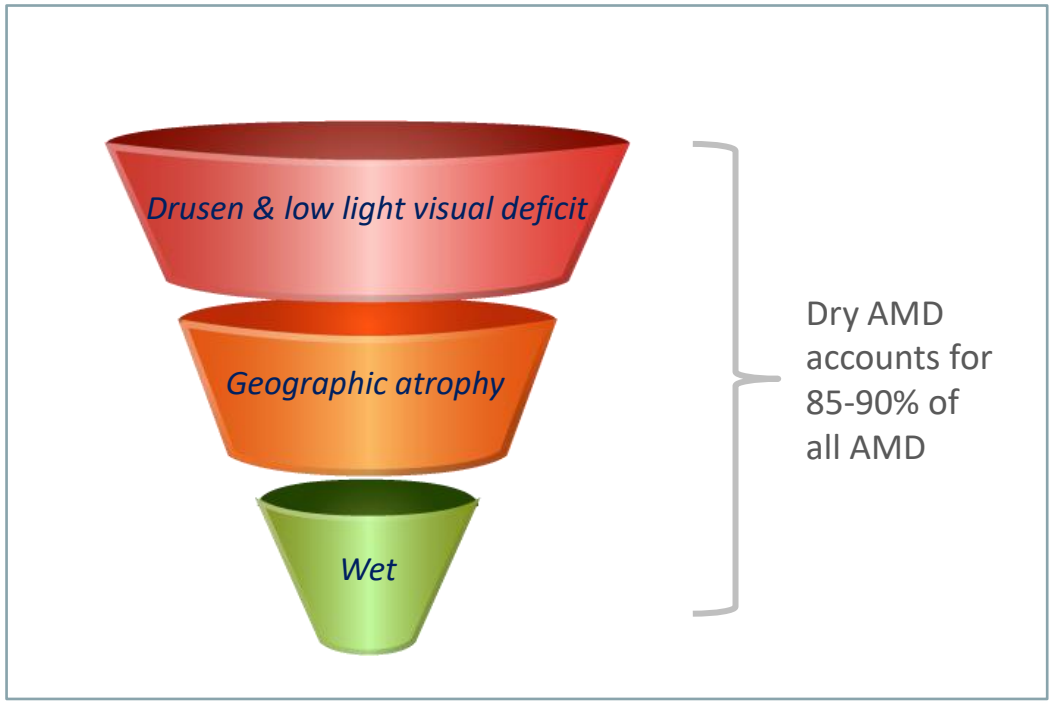
Potential for Early Intervention in Progressive Disease

Late-stage AMD (GA and wet AMD) affects millions of elderly Americans, including >14% of white Americans age ≥80[1]



Macular degeneration affects >10 million Americans[2]



[1] National Eye Institute (https://www.nei.nih.gov)

[2] American Macular Degeneration Foundation (https://www.macular.org)

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NEUROLOGY PLATFORM

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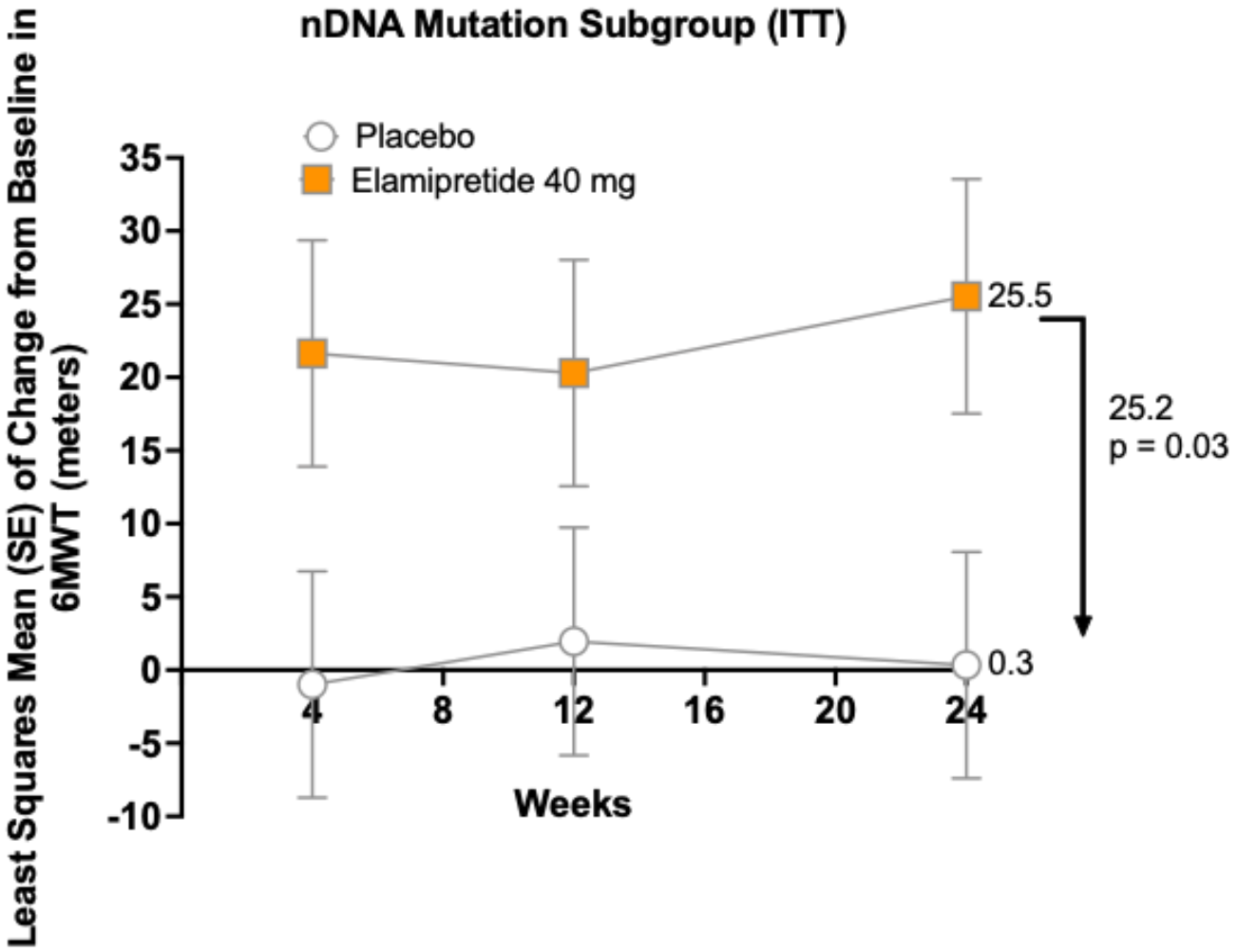
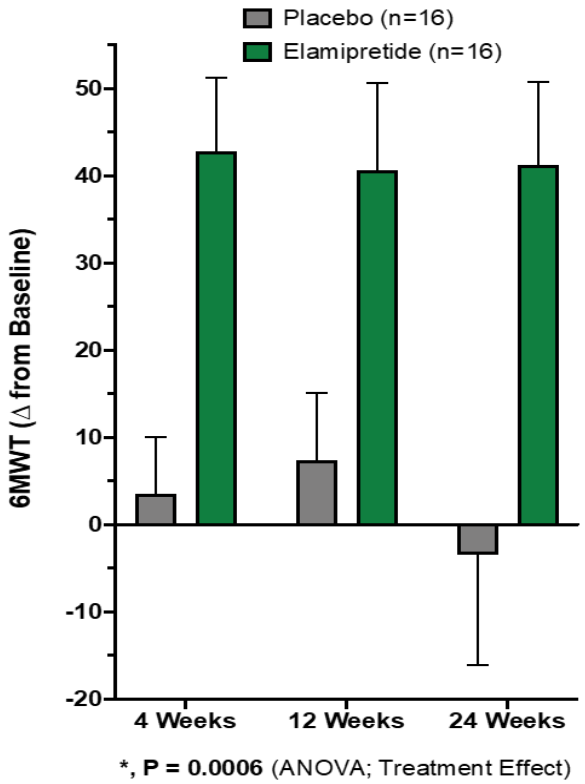
Primary Mitochondrial Disease due to nDNA mutations (nPMD)

Post-hoc Analyses of data in nPMD patients



Enriching for replisome-related nDNA mutations + PEO co-morbidity ensures myopathic phenotype, predicts more robust response



*, **P = 0.0006** (ANOVA; Treatment Effect)

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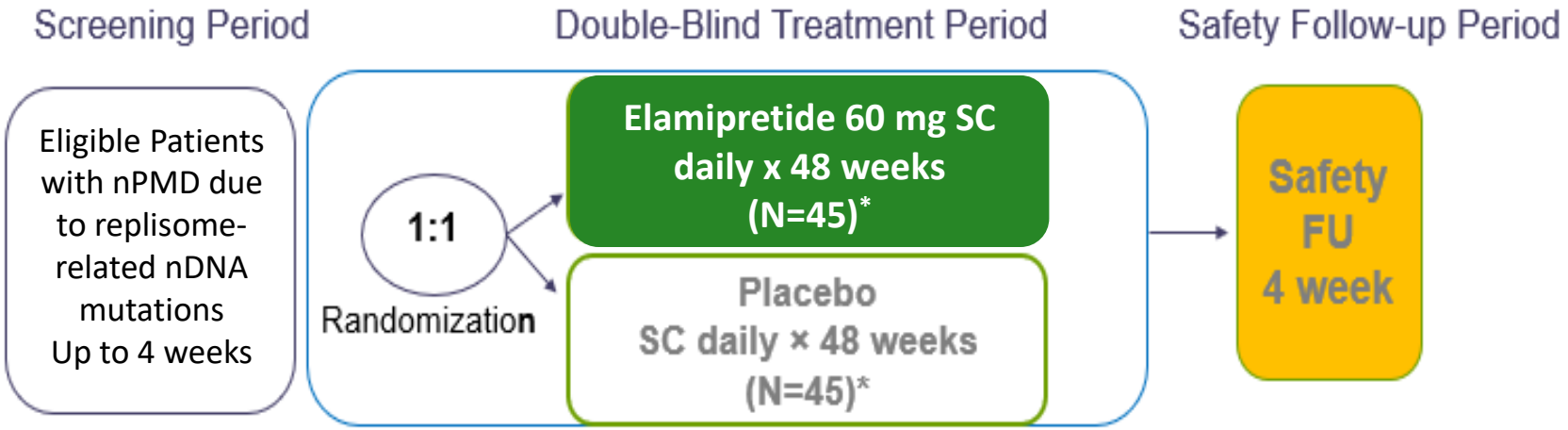
Primary Mitochondrial Disease due to nDNA mutations (nPMD)



* Up to 40 additional patients with non-replisome nDNA mutations

- Type C meeting to discuss P3 protocol scheduled with Division of Rare Disease and Medical Genetics
- Proposing primary efficacy analysis in patients with POLG and other replisome-related mutations (n=90)
- 60 mg SC once-daily
- 6MWT primary endpoint; 5XSST, 3TUG, PROs secondary endpoints
- 1-year duration
- Initiation year-end 2021

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First Quarter 2021 Financial Results

(In Millions)	Three Months Ended March 31	
	2021	2020
Total Revenue	-	-
Total Operating Expenses		
Research and Development	6.1	9.8
General and Administrative	5.0	5.2
Net Loss from Operations	11.1	15.0

We expect our cash and cash equivalents of $32.1 million as of March 31, 2021 and the $30.0 million of additional funding under the Development Agreement expected during 2021 to fund our operations into the second quarter of 2022

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Building a Transformational Foundation for Mitochondrial Medicine

2021

- Start Phase 3 Randomized Withdrawal Trial in Barth Syndrome to Inform Potential 2022 NDA Submission
- Start Phase 2a Investigator-Sponsored Clinical Trial in Friedreich's Ataxia
- Initiate Regulatory Engagements Ahead of Duchenne Cardiomyopathy Trial Initiation
- Prepare for ReCLAIM Phase 2 Read-out by Elucidating Enrichment Strategies and IVT Feasibility
- Start Phase 3 Clinical Trial in Primary Mitochondrial Disease due to nDNA Mutations
- Progress Toward Phase 2 Readiness for SBT-272 Neurology Indication(s)

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Q & A

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