Forward Looking Statements

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Gene Editing Transforms Medicines

Gene edited medicines offer potential cures to patients with severe diseases

Potential for…

☑ Superior efficacy
☑ Treating untreatable diseases
☑ Durable response
☑ One-time curative treatments
☑ Improved safety
☑ Greater precision

Expanding and accelerating the possibility of in vivo and ex vivo genomic medicines
Leader in Genomic Medicine

Only company with a portfolio of proprietary CRISPR Cas9 and Cas12a enzymes to address the widest range of diseases

**In Vivo Gene Edited Medicines**

Leverage smaller *Staph. aureus* Cas9 and AAV delivery technology for enhanced efficiency in ocular, neurological, and future therapeutic areas

**Ex Vivo Gene Edited Cell Medicines**

Leverage Cas12a for enhanced efficiency and editing specificity for hemoglobinopathies and solid tumors

Best-in-class gene editing platform, broadest intellectual property, flexible and robust manufacturing capabilities, led by seasoned executive team
Company Highlights

Best-In-Class In Vivo and Ex Vivo Gene Edited Medicines

First ever administration of an *in vivo* gene editing medicine in humans with EDIT-101 for potentially curing genetic blindness

Expanding *in vivo* gene editing medicines to address unmet monogenetic diseases worldwide

Developing EDIT-301 as potential best-in-class *ex vivo* cell medicine for sickle cell disease and beta-thalassemia

Developing potential best-in-class iPSC-derived NK (iNK) cell medicines for solid tumors

Financial, Operational and Organizational Excellence

Robust internal and external manufacturing capabilities, ready to scale for commercialization

Strong intellectual property position in the space with exclusive rights to foundational Cas9 and Cas12a patent estates

Sufficient capital to sustain operations well into 2023

Diverse and experienced leadership team bridging from research towards commercialization
## Pipeline

<table>
<thead>
<tr>
<th>PROGRAM (OR DISEASE/CANDIDATE)</th>
<th>DISCOVERY</th>
<th>LEAD OPTIMIZATION</th>
<th>IND ENABLING</th>
<th>EARLY-STAGE CLINICAL</th>
<th>LATE-STAGE CLINICAL</th>
<th>COMMERCIAL PARTNER</th>
<th>ENABLING TECHNOLOGY</th>
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<tbody>
<tr>
<td><strong>IN VIVO GENE EDITED MEDICINES</strong></td>
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<td>EDIT-101: Leber Congenital Amaurosis 10 (LCA10)</td>
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<td>EDIT-102: Usher Syndrome 2A (USH2A)</td>
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<td>Autosomal Dominant Retinitis Pigmentosa 4 (RP4)</td>
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<td><strong>OTHER INDICATIONS</strong></td>
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<td>Neurological Diseases</td>
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<td><strong>EX VIVO GENE EDITED CELL MEDICINES</strong></td>
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<td><strong>HEMOGLOBINOPATHIES</strong></td>
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<td>EDIT-301: Sickle Cell Disease (SCD)</td>
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<td>EDIT-301: β-Thalassemia</td>
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<td>αβ T Cells</td>
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<td>iPSC NK (iNK) Cells</td>
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*iPSC: induced pluripotent stem cell*
2021 Anticipated Milestones

**In Vivo Gene Edited Medicines**

**Ocular**
- Initiate dosing of second cohort for Brilliance trial for EDIT-101 in Q1 2021
- Present clinical data for EDIT-101 by year-end
- Declare development candidate for RP4 by year-end

**Other Indications**
- Advance *in vivo* gene edited medicines with AskBio

**Ex Vivo Gene Edited Cell Medicines**

**Hematology**
- Initiate dosing of EDIT-301 for Ruby trial for sickle cell disease
- File IND for EDIT-301 for beta-thalassemia by year-end

**Oncology**
- Advance *ex vivo* preclinical studies for a gene edited iNK cell medicine to treat solid tumors
- Advance αβ T cell medicines in collaboration with Bristol-Myers Squibb
In Vivo Gene Edited Medicines

Potential to address significant unmet need

Future Indications

Over 6,000 human genetic disorders

- Neuromuscular
- Liver
- Hematology
- Central nervous system
- Cardiology
- Other therapeutic areas

Next Indication: Neurology

- Undisclosed neurological indication

Initial Focus: Ocular

5.5 million patients with IRDs worldwide

EDIT-101: Leber congenital amaurosis 10 (LCA10)
EDIT-102: Usher syndrome 2A (USH2A)
RP4 ( Autosomal dominant retinitis pigmentosa 4)
Other inherited retinal diseases
## Ocular Program Overview

<table>
<thead>
<tr>
<th></th>
<th>EDIT-101: LCA-10</th>
<th>EDIT-102: USH2A</th>
<th>RP4</th>
<th>Undisclosed Target</th>
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</thead>
<tbody>
<tr>
<td><strong>Inheritance</strong></td>
<td>Autosomal Recessive</td>
<td>Autosomal Recessive</td>
<td>Autosomal Dominant</td>
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<tr>
<td><strong>Gene</strong></td>
<td>CEP-290</td>
<td>Usherin</td>
<td>Rhodopsin</td>
<td></td>
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<tr>
<td><strong>Mutation</strong></td>
<td>c.2991+1655A&gt;G mutation in intron 26 (IVS26)</td>
<td>Exon 13 mutations</td>
<td>RHO mutations</td>
<td></td>
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<tr>
<td><strong>Target Cells</strong></td>
<td>Photoreceptors</td>
<td>Photoreceptors</td>
<td>Photoreceptors</td>
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<tr>
<td><strong>Presentation</strong></td>
<td>Blindness/ severe visual impairment at or near birth</td>
<td>Loss of peripheral and night vision, eventual legal blindness</td>
<td>Reduced rod function, leading to night blindness, loss of peripheral vision</td>
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</table>

**EDIT-101 progress is de-risking subsequent ocular indications**
EDIT-101 to Treat Leber Congenital Amaurosis 10 (LCA10)
First Ever *In Vivo* Gene Edited Medicine Administered to Humans

**Disease**
- Lack of outer segment of photoreceptors leading to blindness in childhood

**Approach**
- Potentially cure genetic blindness by removing CEP290 genetic mutation in photoreceptors

**Status**
- Initiated dosing of second cohort (adult mid dose) of Brilliance trial
Editing to Correct Vision

LCA10 Photoreceptor

Outer segment absent due to CEP290 deficiency

EDIT-101

Single dose editing removes disease-causing mutation

Rescued Photoreceptor

Outer segment regenerates with CEP290 protein

Proprietary SaCas9 enzyme
EDIT-101 Trial Design, Status & Update

**STATUS**

Initiated dosing of second cohort (adult mid dose)

**PATIENTS**

18 patients, aged 3 years and above

**INTERVENTION**

Single dose of EDIT-101 administered via subretinal injection to eye with worse vision

**ENDPOINTS**

- **Primary: Safety** including frequency and number of adverse events related to drug, procedure, and dose limiting toxicities
- **Secondary: Efficacy** including visual acuity, mobility course, macula thickness, pupillometry, and electroretinogram using patient’s own baseline value for each efficacy measure

**PROTOCOL**

Based on safety in the first cohort, protocol was amended to broaden inclusion criteria of sentinel patients

**SAFETY REVIEW**

IDMC review following treatment of first 2 patients in Cohort 2 to assess start of dosing in pediatric patients
EDIT-102 to Treat Usher Syndrome Type 2A

Disease
Degeneration of photoreceptors causing progressive vision loss and blindness

Approach
Remove USH2A mutation in photoreceptors using same enzyme, AAV, and promoter as EDIT-101

Status
Transferred CMC activities from AbbVie to CDMO
Autosomal Dominant Retinitis Pigmentosa 4 (RP4)

**Disease**
Progressive decline in night vision, followed by peripheral vision, and eventual blindness

**Approach**
Replace rhodopsin gene in photoreceptors to correct all RP4 mutations with AAV

**Status**
Declare development candidate by year end 2021
Tremendous Opportunity for Unmet Inherited Retinal Diseases

Estimated Patient Population

<table>
<thead>
<tr>
<th></th>
<th>U.S.</th>
<th>E.U.</th>
<th>Rest of World</th>
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<tbody>
<tr>
<td>EDIT-101: LCA-10</td>
<td>1,500</td>
<td>2,500</td>
<td>30,000+</td>
</tr>
<tr>
<td>EDIT-102: USH2A</td>
<td>2,000</td>
<td>2,700</td>
<td>40,000+</td>
</tr>
<tr>
<td>RP4</td>
<td>8,000</td>
<td>10,700</td>
<td>150,000+</td>
</tr>
</tbody>
</table>

Global Market Potential

- Future In Vivo Indications
- RP4 and Other Inherited Retinal Diseases
- LCA10 and USH2A

Global Market Potential ($)

Patient populations compiled from various sources.
EDIT-101 and EDIT-102 patient populations for specific targeted mutations.
Ex Vivo Gene Edited Cell Medicines for Hemoglobinopathies
Potential Best-in-Class Medicine for Sickle Cell Disease and Beta-Thalassemia

Use proprietary Cas12a enzyme to edit beta-globin locus to safely, robustly, and durably increase fetal hemoglobin with single administration

- More precise genomic alterations than lentiviral gene therapy
- Reduces sickle globin, in contrast to lentiviral gene therapy
- Editing beta-globin locus provides level of inherent safety, in contrast to editing at the BCL11A site
- More robustly repopulates red blood cell lineage than editing at the BCL11A site
- Demonstration of no measurable off-targets

**Epidemiology**

**Sickle Cell:**
165,000+ sickle cell patients and 15,000+ beta-thalassemia patients in the U.S. and Europe\(^1,2,3\)

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\(^1\) National Center on Birth Defects and Developmental Disabilities, Centers for Disease Control and Prevention
\(^2\) European Medicines Agency: EU/3/19/2242
\(^3\) DRG: Rare Diseases and Orphan Drugs, Oct 15, 2020
EDIT-301 to Treat Sickle Cell Disease and Beta-Thalassemia

**Disease**
Deformed and diminished blood cells causing anemia, pain crises, organ failure, and mortality

**Approach**
Leverage proprietary Cas12a enzyme to edit β-globin locus to increase fetal hemoglobin

**Status**
Cleared to initiate dosing sickle cell patients*
Planning to submit IND for β-thalassemia by year-end

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* The U.S. Food and Drug Administration (FDA) has cleared the initiation of the safety phase of the Company’s EDIT-301 clinical trial, and the Company can begin dosing patients. The Company is required to develop and submit to the FDA an improved potency assay prior to enrolling the efficacy phase of the RUBY trial.
EDIT-301 Process

**STEM CELL MOBILIZATION**
Administer mobilization agent to increase production of stem cells and drive them into peripheral blood.

**COLLECTION**
Collect blood from patient and enrich nucleated cells via leukapheresis.

**MYELOABLATION**
Myeloablation with busulfan to remove diseased cells for replacement with edited cells.

**PROCESSING**
Collection sent to processing facility, with portion retained at clinical trial site for back-up/rescue.

**EDITING**
Hematopoietic stem cells edited at beta-globin locus with CRISPR/Cas12a.

**EDIT-301 REINFUSION**
Infuse EDIT-301 into peripheral blood.
Goal is Superior Safety and Efficacy

Proprietary Cas12a editing at the HBG1/2 promoter overcomes shortcomings of other treatments

<table>
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<tr>
<th></th>
<th>EDIT-301: β-globin Locus</th>
<th>BCL11A Editing</th>
<th>Lentiviral Gene Therapy</th>
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<tr>
<td><strong>EFFICACY</strong></td>
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<tr>
<td>Directly upregulates fetal hemoglobin?</td>
<td>✓</td>
<td>✗</td>
<td>✗</td>
</tr>
<tr>
<td>Reduces sickling?</td>
<td>✓</td>
<td>✓</td>
<td>✗</td>
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<tr>
<td><strong>SAFETY</strong></td>
<td></td>
<td></td>
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<tr>
<td>Precise editing at specified location in genome?</td>
<td>✓</td>
<td>✓</td>
<td>✗</td>
</tr>
<tr>
<td>Targets natural locations of fetal hemoglobin mutations?</td>
<td>✓</td>
<td>✗</td>
<td>✗</td>
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</table>
Large Scale Manufacturing in Place for EDIT-301

No lineage skewing after engraftment

Stable polyclonal engraftment

Female NSG mice bone marrow 20 weeks post-infusion

n=46–48 mice/treatment

Unedited CD34+ cells
Edited CD34+ cells

Blood draws over 20 weeks

20 weeks post-infusion
Ex Vivo Gene Edited Cell Medicines for Oncology
Potential Best-in-Class NK Medicines for Solid Tumors

Gene edited iPSC cells are revolutionizing cancer therapy through numerous cellular advantages

- Unlimited self-renewal potential
- Single cell clonability for generating homogeneous cell population
- Cas12a edited antigen-specific cells for targeted therapy
- Fully characterized cell line
- Potential for developing cryopreserved, off-the-shelf therapeutic products

Epidemiology

Over 1.3 million new cases of solid tumor cancers, linked to over 400,000 deaths, per year in the US

1 American Cancer Society: Cancer Facts & Figures 2020
Gene Edited iPSC NK (iNK) Cell Medicines to Treat Solid Tumors

Disease
Malignant solid tumors that develop in lung, colon, breast, and other organs

Approach
Multiplexed gene editing enhanced tumor-killing, off-the-shelf cell therapies

Status
Advancing preclinical studies for development candidate
Gene Edited iNK Cells Will Be Transformational for Cancer Patients

Advantages of NK Cells

Naturally allogeneic because they do not express T cell receptors

Rapidly recognize broader array of tumors with stress ligand receptors

Recognize tumors lacking MHC I that evade T cells and therapeutic antibodies

Recruit T cells to kill tumors by releasing IFN-γ and TNF-α

Directly kill tumors by releasing granzyme and perforin

Edited iPSC-derived NK Cell

KO1: Overcome resistance with TGFβR2 knockout

KO2: Improve persistence with CISH knockout

Undisclosed Edit

MHC I: major histocompatibility complex class 1; IFN-γ: interferon-gamma; TNF-α: tumor necrosis factor-alpha
Efficient Editing and Sustained Anti-Tumor Activity

Efficient knock out of multiple genes in iNKs

Greater reduction of SK-OV-3 spheroid size

Editing Efficiency (%)

KO 1 KO 2 KO 3 KO 4 KO 5

DNA harvested from cells 4 days after electroporation

Greater reduction of SK-OV-3 spheroid size

Unedited iNK cells (n=6)

CISH⁻/TGFB2⁻ iNK cells (n=6)

Tumor size reduced by 87%

Presented at ASH 2020
*p<0.05; **p<0.01; ***p<0.001 vs unedited iNK cells (two-way ANOVA, Sidak’s multiple comparisons test)
Collaborations

**DEVELOPMENT & COMMERCIALIZATION**

- **αβ T cell medicines to treat cancer and autoimmune diseases**
- $100 million in payments to date plus potential for milestones and tiered royalties

**ENABLING TECHNOLOGY**

- **askbio**
  - Collaboration bringing leading capsid development, clinical stage AAV vector delivery system, and manufacturing expertise

**IN VIVO GENE EDITED MEDICINES**

- Collaboration to create novel, allogeneic pluripotent stem cell (PSC) lines using BlueRock’s induced pluripotent stem cell (iPSC) platform to develop CRISPR iNK cell medicines

**GENE EDITED αβ T CELL MEDICINES FOR CANCER**

- Bristol Myers Squibb

**GENE EDITED iNK CELL MEDICINES FOR CANCER**

- BlueRock Therapeutics
Internal & External Manufacturing Capabilities

Boulder, CO
- cGMP Guide RNA manufacturing for Editas and partner programs.

Greater Boston
- Multi-year lease cGMP manufacturing facilities staffed by Editas personnel to support preclinical and early-phase clinical cell manufacturing activities.

Strategic Global Partnership
- Leveraging multi-national footprint to manufacture gene and cell therapies.

Cleanrooms on Demand™
Intellectual Property

**Unmatched intellectual property portfolio in CRISPR gene editing**

- **Foundation**
  Exclusive foundational IP for CRISPR/ Cas9 and Cas12a (Cpf1) editing in human therapeutics

- **Breadth**
  Multiple species and CRISPR forms to address widest range of diseases

- **Depth**
  Over 220 issued patents, over 800 applications pending

- **Markets**
  Global coverage including US, Europe, Japan, Australia, Canada, China
Executive Team

James C. Mullen
Chief Executive Officer

Michelle Robertson
Chief Financial Officer

Lisa A. Michaels, M.D.
Chief Medical Officer

Charlene Stern, Ph.D., J.D.
Chief Legal Officer

Gad Berdugo
Chief Business Officer

Harry Gill
Senior Vice President, Operations

Clare Carmichael
Chief Human Resources Officer

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Company Highlights

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