Overview of Development Process

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October 6, 2018

Forward Looking Statements

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Developing Best-in-Class CRISPR Medicines

Ocular Medicines

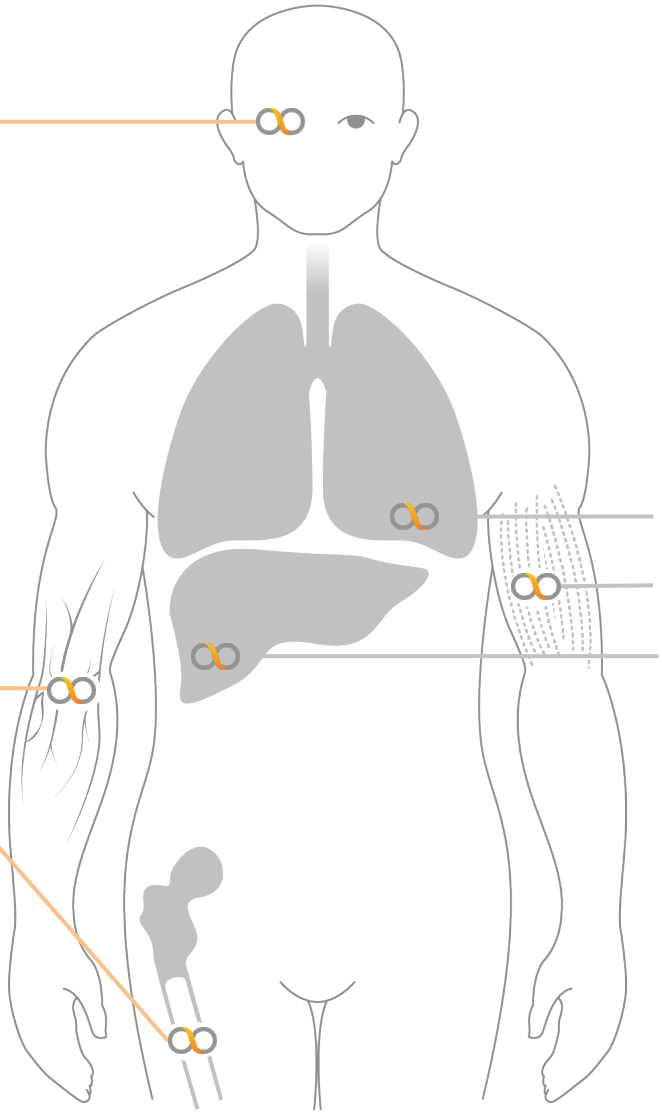
Inherited Retinal Diseases
- LCA10 (EDIT-101)*
- USH2A
- Additional unnamed targets

Infectious Diseases
- Ocular HSV

Engineered Cell Medicines

Immune Cells
- T Cells – Cancer**
- T Cells – Autoimmune diseases

Stem Cells
- HSCs – Sickle Cell Disease
- HSCs – Beta-thalassemia



Early Discovery
- Lung – CF
- Muscle – DMD
- Liver – AATD

in vitro proof-of-concept

in vivo proof-of-concept

*Partnered with Allergan – US 50/50 plus milestones and ex-US royalties; **Partnered with Celgene – global milestones and royalties; LCA10: Leber Congenital Amaurosis Type 10; USH2A: Usher Syndrome Type 2A; HSV: Herpes Simplex Virus; CF: Cystic Fibrosis; DMD: Duchenne Muscular Dystrophy; AATD: Alpha-1 Antitrypsin Deficiency; HSC: Hematopoietic Stem Cell

From Gene to Genomic Medicine for LCA10

Many questions to answer in order to advance treatments from concept to human clinical trials.

1. DOES EDITING RESTORE PROTEIN EXPRESSION IN PATIENT CELLS?
2. CAN WE EDIT TARGET CELLS IN BEST PRECLINICAL MODEL ANIMAL?
3. DOES PRODUCT CANDIDATE ACHIEVE THERAPEUTIC EDITING IN HUMAN TISSUE?
4. DOES PRODUCT CANDIDATE HAVE SPECIFICITY FOR HUMAN TESTING?
5. WHAT ARE BEST CLINICAL TRIALS TO PROVE VALUE FOR PATIENTS?

EDIT-101 Advancing Towards Clinical Trials

Pre-clinical research conducted to answer these questions:

1	EDITING APPROACH RESTORES FULL LENGTH CEP290 mRNA AND PROTEIN	Demonstrated in cells from LCA10 patients
2	PREDICTED THERAPEUTIC EDITING ACHIEVED IN NON-HUMAN PRIMATES	Estimated productive editing in primate photoreceptors *in vivo*[1] | Delivery vehicle specifically targets photoreceptors
3	PREDICTED THERAPEUTIC EDITING ACHIEVED IN HUMAN RETINA	Productive editing in human retinal explant photoreceptors[1] | Targeted transduction of photoreceptors
4	COMPREHENSIVE METHODS TO IDENTIFY EFFICIENT AND SPECIFIC GUIDE RNAs	Proprietary computational, biochemical, and cellular approaches

EDIT-101 Advancing Towards Clinical Trials

5 SETTING THE STAGE FOR INTERVENTIONAL TRIALS

Ongoing Natural History Study

Patients	~40 patients, aged 3 and above
Objectives	Characterize patients, assessments, and rate of change and validate endpoints
Sites	6 to 8 sites in US and Europe
Follow-up	6 visits over 1 year

EDIT-101 Advancing Towards Clinical Trials

PHASE 1/2 TRIAL DESIGN IN DEVELOPMENT

Design	Open-label, dose escalation
Patients	~10 to 20 patients with IVS26 mutation
Comparator	Non-randomized comparison to natural history, contralateral eye, and patient baseline
Duration	1 year evaluation of efficacy and safety

IVS26 mutation: Intervening Sequence 26 in CEP290 gene containing the c.2991+1655A>G mutation

Community

Resilience

Ingenuity

Science

Passion

Revolution

Thank You

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