MORGAN L. MAEDER, PhD

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I am a passionate and skilled molecular and cell biologist with over 10 years of experience in the fields of gene editing and gene therapy. I have been successful in directly building my own groups, as well as in leading cross-functional internal teams, and managing external collaborations. I am highly skilled at creative thinking and problem solving and have demonstrated success at implementing novel technologies to understand and solve complex biological questions. At Editas, I have led the first company program to enter the clinic from inception through IND-enabling studies. I am uniquely positioned to be able to lead programs requiring both a strong understanding of technical details, while also maintaining a broad view with an eye towards clinical development.

RESEARCH EXPERIENCE

Third Rock Ventures NewCo, Boston MA

An early-stage, pre-launch company exploring the biology of phase separation and biomolecular condensation to develop therapeutic approaches for a wide range of human diseases.

Consultant October 2018-Present

- Explore relevant biology to determine scientific scope of the company, identify and vet specific targets and build a vision of the company product engine
- Establish the lab and initiate research programs
- Build a strong team through collaborative efforts with academic co-founders, Third Rock Ventures partners and associates, scientists and consultants
- Aid in developing long range scientific vision and value creation plan to prepare company for launch

Editas Medicine, Cambridge MA

As a leading genome editing company, Editas Medicine is focused on using CRISPR/Cas9 technology to develop transformative medicines for the treatment of genetic diseases. Founded in November 2013, the company has active preclinical programs in the areas of inherited retinal disease, hemoglobinopathies and immuno-oncology.

Sr. Scientist August 2018-October 2018
Scientist III January 2016-July 2018
Scientist I January 2014-January 2016
Consultant October-December 2013

- I joined the company as its first scientist and help to set up the lab, conceive, and get the first research programs off the ground. I was responsible for establishing the lab and training other early scientists not familiar with genome editing. I was actively involved in defining the core values and growing the company from an early start-up to a mature public company which is advancing its first program into the clinic.
- Led a research group consisting of 2 research associates and 1 PhD scientist.
- As the LCA10 Program Lead, I played a critical role in the development of EDIT-101 for the treatment of Leber Congenital Amaurosis, an inherited retinal disease caused by mutations in the *CEP290* gene.
    - Developed editing strategy and performed *in vitro* proof of concept studies.
    - Worked with multi-disciplinary team to develop and implement pre-clinical *in vivo studies* (mouse and non-human primate) to demonstrate therapeutic gene editing strategy.
    - Established NGS-based assays for specificity profiling of targeted nucleases.
    - Drafted application for EMA Orphan Medicinal Product Designation, and presented program, in person, at the EMA to obtain Orphan status.
    - Worked with CMC to transfer research protocols and materials to enable GMP manufacturing.
    - Worked with Regulatory to draft materials for, and participate in conversations with, the FDA.
- Led a cross-functional team coordinating efforts from Business Development, Platform Technology, Pharmacology and Histology to explore novel delivery technologies for ocular gene editing.
- Led multiple research programs with external collaborators from Massachusetts Eye and Ear Infirmary, The University of Florida, The University of Alabama at Birmingham and Lions Eye Institute.
- Additionally, my group works to explore and develop editing strategies for additional inherited retinal diseases, and develop complex *in vitro* model systems to interrogate gene editing in photoreceptors.

Harvard University, Massachusetts General Hospital, Charlestown, MA

Advisor: J. Keith Joung

Postdoctoral Associate May-December 2013
PhD Candidate September 2008-May 2013

- Demonstrated first ZFN-induced correction of a disease-causing mutation in human induced pluripotent stem (iPS)cells. Corrected the sickle cell causing mutation in iPS cells derived from sickle cell patients.
- Demonstrated high efficacy and multiplex capabilities of TALE-activator fusions for targeted gene activation.
- Demonstrated first use of CRISPR/Cas9 system for targeted gene activation in human cells.
- Developed ZFN and TALE based systems for targeted DNA demethylation by engineering

fusions to the Tet1 catalytic domain.
- Trained and oversaw the experiments of 3 research technicians reporting directly to me.
- Led multiple collaborations with other academic labs.

Massachusetts General Hospital, Charlestown, MA

Principal Investigator: J. Keith Joung

Research Technologist June 2007 - August 2008
Research Technician June 2006 - June 2007

- Developed and validated a technology platform for engineering designer DNA-binding zinc finger proteins.
- Induced targeted alteration of endogenous genes in human cells using engineered zinc finger nucleases.
- Demonstrated use of combinatorial zinc finger transcription factor libraries for functional genomics studies in human cells.

EDUCATION

PhD in Genetics, **Harvard University**, Cambridge, MA May 2013

Graduate School of Arts and Sciences, Division of Medical Sciences

BA in Biological Sciences, **Connecticut College,** New London, CT May 2006

PROFESSIONAL ACTIVITIES

- American Society of Gene and Cell Therapy, Member (2007-Present)
  - Gene Editing Committee Member (2016-Present)
  - New Investigator Committee Member (2017-Present)
- Association for Research in Vision and Ophthalmology, Member (2015-Present)
- Guest lecturer in Gene Editing course for Harvard Biological and Biomedical Sciences PhD program
- Member of *Human Gene Therapy Methods* Editorial Board
- Peer Reviewer for *Nature Biotechnology, Nature Methods, Nature Communications, Molecular Therapy, Nucleic Acids Research, Nucleic Acids Research Methods* and *PLOS One*

SELECTED PATENT APPLICATIONS

Inventor on total of 15 submitted patent applications and 3 granted patents.

- Crispr/cas-related methods and compositions for treating leber's congenital amaurosis 10 (lca10). US-9938521-B2
- Crispr/cas-related methods and compositions for treating usher syndrome and

retinitis pigmentosa. WO-2015134812-A1
- Compositions and methods for treating cep290 associated disease. WO-2018026976-A1
- Zinc finger nuclease for the CFTR gene and methods of use thereof. US-8846578-B2
- TAL-Tet1 fusion proteins and methods of use thereof. US-9890364-B2

SELECTED PUBLICATIONS

Author on total of 33 research articles, 2 reviews and 5 methods papers/book chapters

Maeder ML*, Stefanidakis M, Wilson CJ, Baral R, Barrera LA, Bounoutas GS, Bumcrot D, Chao H, Ciulla DM, DaSilva JA, Dass A, Dhanapal V, Fennell TJ, Friedland AE, Giannoukos G, Gloskowski SW, Glucksmann A, Gotta GM, Jayaram H, Haskett SJ, Hopkins B, Horng JE, Joshi S, Marco E, Mepani R, Reyon D, Ta T, Tabbaa DG, Samuelsson SJ, Shen S, Skor MN, Stetkiewicz P, Wang T, Yudkoff C, Myer VE, Albright CF, Jiang H. Development of a gene-editing approach to restore vision loss in Leber congenital amaurosis type 10. Nat Med. 2019 Jan 21.
***Corresponding Author**

McCullough KT, Boye SL, Fajardo D, Calabro K, Peterson JJ, Strang CE, Chakraborty D, Gloskowski S, Haskett S, Samuelsson S, Jiang H, Witherspoon CD, Gamlin PD, **Maeder ML**, Boye SE. Somatic Gene Editing of GUCY2D by AAV-CRISPR/Cas9 Alters Retinal Structure and Function in Mouse and Macaque. Hum Gene Ther. 2018 Dec 20.

Wilson CJ, Fennell T, Bothmer A, **Maeder ML**, Reyon D, Cotta-Ramusino C, Fernandez CA, Marco E, Barrera LA, Jayaram H, Albright CF, Cox GF, Church GM, Myer VE. Response to "Unexpected mutations after CRISPR-Cas9 editing in vivo". *Nat Methods*. 2018 Apr;15(4):236-237.

Giannoukos G, Ciulla DM, Marco E, Abdulkerim HS, Barrera LA, Bothmer A, Dhanapal V, Gloskowski SW, Jayaram H, **Maeder ML**, Skor MN, Wang T, Myer VE, Wilson CJ. UDiTaS™, a genome editing detection method for indels and genome rearrangements. *BMC Genomics*. 2018 Mar 21;19(1):212.

Maeder ML, Gersbach CA. Genome-editing Technologies for Gene and Cell Therapy. *Molecular Therapy*. 2016 Mar;24(3):430-46.

Friedland AE, Baral R, Singhal P, Loveluck K, Shen S, Sanchez M, Marco E, Gotta GM, **Maeder ML**, Kennedy EM, Kornepati AV, Sousa A, Collins MA, Jayaram H, Cullen BR, Bumcrot D. Characterization of Staphylococcus aureus Cas9: a smaller Cas9 for all-in-one adeno-associated virus delivery and paired nickase applications. *Genome Biol*. 2015 Nov 24;16:257.

Maeder ML*, Angstman JF*, Richardson ME, Linder SJ, Cascio VM, Tsai SQ, Ho QH, Sander JD, Reyon D, Bernstein BE, Costello JF, Wilkinson MF, Joung JK. Targeted DNA demethylation and activation of endogenous genes using programmable TALE-TET1 fusion proteins. *Nature Biotechnology*. 2013 Dec;31(12):1137-42.

Maeder ML, Linder SJ, Cascio VM, Fu Y, Ho QH, Joung JK. CRISPR RNA-guided activation of endogenous human genes. *Nature Methods*. 2013 Oct;10(10):977-9.

Maeder ML, Linder SJ, Reyon D, Angstman JF, Fu Y, Sander JD, Joung JK. Robust, synergistic regulation of human gene expression using TALE activators. *Nature Methods*. 2013 Mar;10(3):243-5.

Hwang WY, Fu Y, Reyon D, **Maeder ML**, Tsai SQ, Sander JD, Peterson RT, Yeh JR, Joung JK. Efficient genome editing in zebrafish using a CRISPR-Cas system. *Nature Biotechnology*. 2013 Mar;31(3):227-9.

Sebastiano V*, **Maeder ML***, Angstman JF, Haddad B, Khayter C, Yeo DT, Goodwin MJ, Hawkins JS, Ramirez CL, Batista LF, Artandi SE, Wernig M, Joung JK. In situ genetic correction of the sickle cell anemia mutation in human induced pluripotent stem cells using engineered zinc finger nucleases. *Stem Cells*. 2011 Nov;29(11):1717-26.

Zou J, **Maeder ML**, Mali P, Pruett-Miller SM, Thibodeau-Beganny S, Chou BK, Chen G, Ye Z, Park IH, Daley GQ, Porteus MH, Joung JK, Cheng L. Gene targeting of a disease-related gene in human induced pluripotent stem and embryonic stem cells. *Cell Stem Cell*. 2009 Jul 2; 5(1):97-110.

Townsend JA, Wright DA, Winfrey RJ, Fu F, **Maeder ML**, Joung JK, Voytas DF. High-frequency modification of plant genes using engineered zinc-finger nucleases. *Nature*. 2009 May 21; 459(7245):442-5.

Maeder ML, Thibodeau-Beganny S, Osiak A, Wright DA, Anthony RM, Eichtinger M, Jiang T, Foley JE, Winfrey RJ, Townsend JA, Unger-Wallace E, Sander JD, Müller-Lerch F, Fu F, Pearlberg J, Göbel C, Dassie JP, Pruett-Miller SM, Porteus MH, Sgroi DC, Iafrate AJ, Dobbs D, McCray PB Jr, Cathomen T, Voytas DF, Joung JK. Rapid "open-source" engineering of customized zinc-finger nucleases for highly efficient gene modification. *Mol Cell*. 2008 Jul 25; 31(2):294-301.

SELECTED PRESENTATIONS

Efficient *In Vivo* Gene Editing of Inherited Retinal Disease Genes in Mice and Non-Human Primates. Oral Abstract. American Society of Gene and Cell Therapy 20th Annual Meeting. Washington DC. May 2017.

Envisioning a Gene Editing Approach to Treat Inherited Blindness. Invited Speaker. ProRetina Society Annual Meeting. Potsdam, Germany. April 2017.

Envisioning a Gene Editing Approach to Treat Inherited Blindness. Invited Speaker. FASEB Research Conference – Genome Engineering: Cutting Edge Research and Applications. Lisbon, Portugal. June 2016.

Therapeutic Correction of an LCA-Causing Splice Defect in the CEP290 Gene by CRISPR/Cas-Mediated Genome Editing. Oral Abstract. American Society of Gene and Cell Therapy 18th Annual Meeting. Washington, DC. May 2015.