



MEIRAGTx

Barclays Global Healthcare Conference

March 12, 2020

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Forward Looking Statements

Forward-Looking Statements

This presentation contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. All statements contained in this presentation that do not relate to matters of historical fact should be considered forward-looking statements, including, without limitation, statements regarding our product candidate development and anticipated 2020 milestones regarding our pre-clinical and clinical data and reporting of such data and the timing of results of data, as well as statements that include the words “expect,” “intend,” “plan,” “believe,” “project,” “forecast,” “estimate,” “may,” “should,” “anticipate” and similar statements of a future or forward-looking nature. These forward-looking statements are based on management’s current expectations. These statements are neither promises nor guarantees, but involve known and unknown risks, uncertainties and other important factors that may cause actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements, including, but not limited to, our incurrence of significant losses; any inability to achieve or maintain profitability, acquire additional capital, identify additional and develop existing product candidates, successfully execute strategic priorities, bring product candidates to market, expansion of our manufacturing facilities and processes, successfully enroll patients in and complete clinical trials, accurately predict growth assumptions, recognize benefits of any orphan drug designations, retain key personnel or attract qualified employees, or incur expected levels of operating expenses; failure of early data to predict eventual outcomes; failure to obtain FDA or other regulatory approval for product candidates within expected time frames or at all; the novel nature and impact of negative public opinion of gene therapy; failure to comply with ongoing regulatory obligations; contamination or shortage of raw materials or other manufacturing issues; changes in healthcare laws; risks associated with our international operations; significant competition in the pharmaceutical and biotechnology industries; dependence on third parties; risks related to intellectual property; changes in tax policy or treatment; our ability to utilize our loss and tax credit carryforwards; litigation risks; and the other important factors discussed under the caption “Risk Factors” in our Annual Report on Form 10-K for the year ended December 31, 2019, as such factors may be updated from time to time in our other filings with the SEC, which are accessible on the SEC’s website at www.sec.gov. These and other important factors could cause actual results to differ materially from those indicated by the forward-looking statements made in this press release. Any such forward-looking statements represent management’s estimates as of the date of this press release. While we may elect to update such forward-looking statements at some point in the future, unless required by law, we disclaim any obligation to do so, even if subsequent events cause our views to change. Thus, one should not assume that our silence over time means that actual events are bearing out as expressed or implied in such forward-looking statements. These forward-looking statements should not be relied upon as representing our views as of any date subsequent to the date of this presentation. Unless otherwise stated or the context otherwise requires, the information herein is as of March 11, 2020.

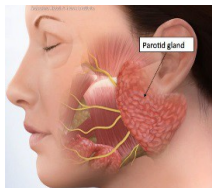
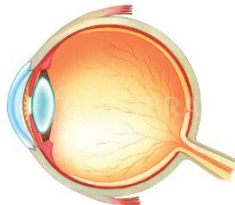
A Vertically Integrated, Clinical Stage Gene Therapy Company

Developing a new pharmaceutical modality designed for the cost effective treatment of a broad range of serious disorders

Diversified Pipeline of Gene Therapy Candidates

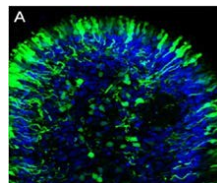
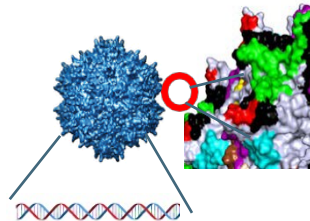
6 ongoing clinical programs:

- Inherited retinal diseases
- Salivary gland
- Parkinson's Disease



Platform of Core Viral Vector Engineering Capabilities

Viral vector design,
promoters, capsid,
transgene optimization,
process development
expertise



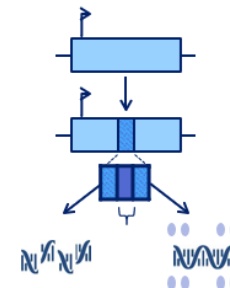
Manufacturing Capacity & Know-How

Flexible and scalable cGMP manufacturing facility with
capacity for commercial
supply for our programs



Next Generation Gene Therapy Riboswitch-Based Gene Regulation

Proprietary technology that
may allow for innovative
gene therapy treatments
whose **expression can be**
turned on and off with an
easily administered small
molecule



cGMP Certified Manufacturing Facility: Flexible and Scalable

Central London facility

- cGMP certified 29,000 sq. ft multi-product, multi-viral vector manufacturing facility
- Designed to meet MHRA, EMA and FDA regulatory requirements
- Single use philosophy / fully enclosed technologies
- 2 cell suites; 3 viral vector suites
- Independent air handling
- Designed for minimal downtime and maximum flexibility
- Adherent / non-adherent cell lines – HEK293
- Support laboratories: Quality Control
- Adjacent MSAT (Manufacturing Science and Technology) area/pilot plant

Expanding manufacturing footprint

- Construction on 2nd cGMP viral vector manufacturing facility & cGMP plasmid production facility expected to begin in 2020



Multiple Therapeutic Targets



OCULAR

Clinical Development

- IRD franchise: XLRP, achromatopsia, *RPE65*-associated retinal dystrophy, LCA4

Research

- Wet AMD, Dry AMD

Gene Regulation

- VEGFR2 Ab – eye drops



NEURODEGENERATIVE

Clinical Development

- Parkinson's disease

Research

- Amyotrophic Lateral Sclerosis (ALS)

Gene Regulation

- CNS expression with BBB penetrant small molecules



SALIVARY GLAND

Clinical Development

- Radiation-induced xerostomia (Grade 2/3)

Research

- Sjogren's Syndrome




Gene Regulation

- Peptide and hormone salivary gland delivery

Human proof of concept demonstrated across ocular, neurodegenerative and salivary gland pipelines

Vector development & optimization technology create opportunities to treat broader indications beyond rare, inherited genetic disorders

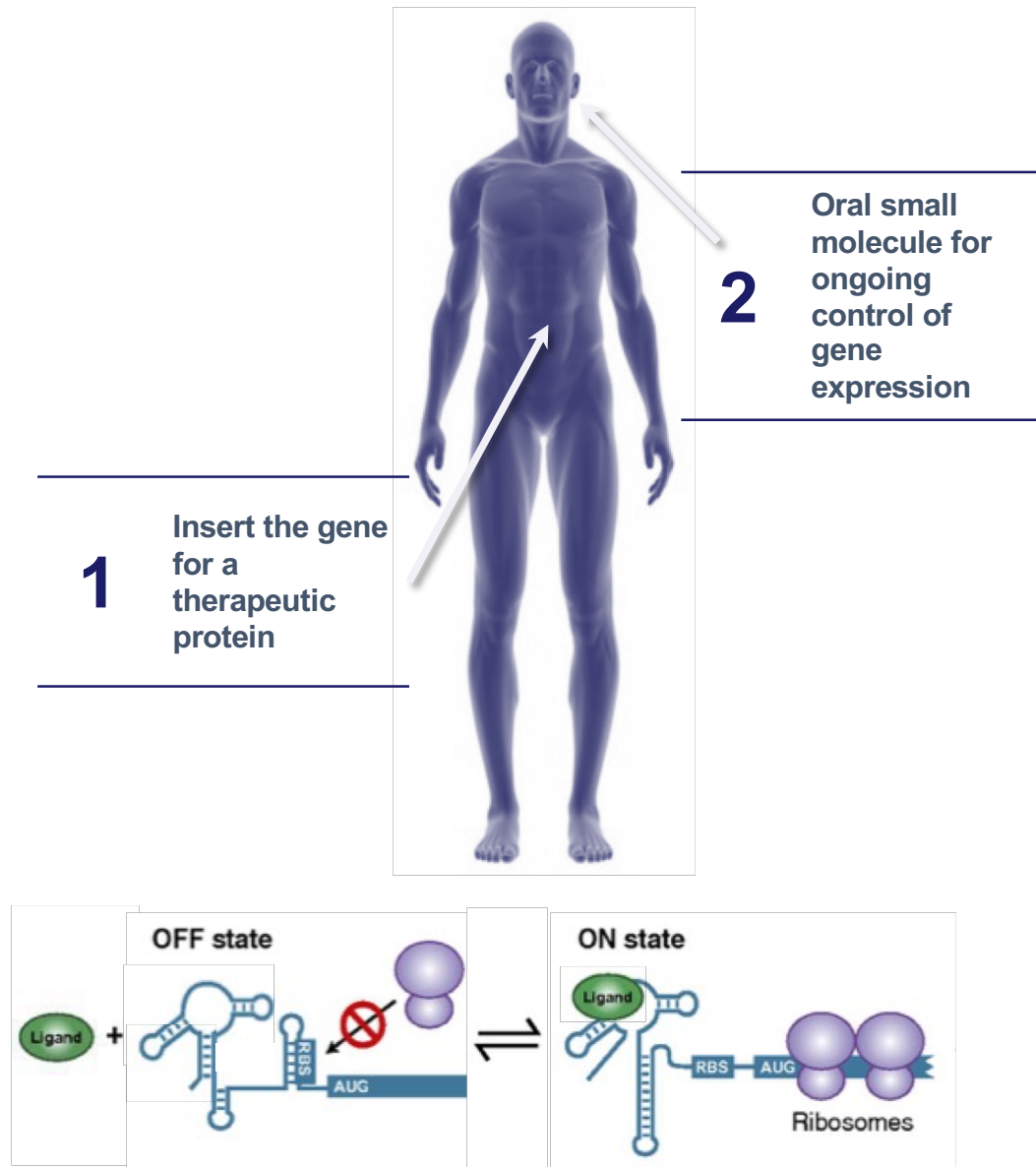
Broad Clinical Pipeline

Product	Indication	Preclinical	Phase 1/2	Details
Ocular				
AAV-RPE65	RPE65-Deficiency	RPDD, Orphan Drug		
AAV-CNGB3*	Achromatopsia (CNGB3)	RPDD, PRIME, Fast Track, Orphan Drug		janssen 
AAV-RPGR*	X-linked RP (RPGR)	PRIME, Fast Track, Orphan Drug		janssen 
AAV-CNGA3*	Achromatopsia (CNGA3)	RPDD, Orphan Drug		janssen 
AAV-AIPL1	LCA4 (AIPL1)	Orphan U.S. & EU		Compassionate use under MHRA Specials License
A006	Wet AMD (anti-VEGFR2)			
Neurodegenerative Disease				
AAV-GAD	Parkinson's Disease (GAD)			
Salivary Gland				
AAV-AQP1	Xerostomia (hAQP1)	Orphan Drug		Phase 1 study at NIH ongoing; multi-site Phase 1/2 trial ongoing
AAV-AQP1	Sjögren's Syndrome (hAQP1)			

*Co-development program with Janssen Pharmaceuticals pursuant to a collaboration agreement.

Next Generation Gene Therapy: Gene Regulation Platform

- **Modular switch cassette based on RNA shape**
- Regulate a chosen transgene in vivo using a different **small molecule** for each transgene
- **Platform can regulate multiple genes:** antibodies, hormones, cytokines
- **Demonstrated regulation in vivo in the liver of AAV delivered genome**



Anticipated Key 2020 Milestones

Ocular

AAV-RPGR for the treatment of XLRP

- Report data from ongoing Phase 1/2 trial in 2020

Salivary gland

AAV-AQP1 for the treatment of Grade 2/3 radiation-induced xerostomia

- Report preliminary data from ongoing Phase 1/2 trial in the second half of 2020

Neurodegenerative disease

AAV-GAD for the treatment of Parkinson's disease

- File IND in the second half of 2020 in order to initiate next clinical trial of AAV-GAD

Manufacturing

Second GMP viral vector manufacturing facility

- Initiate construction mid-2020

GMP plasmid production facility

- Operational by year-end 2020



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Fireside Chat with Gena Wang, Ph.D. Barclays Biotech Equity Research



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