

DEVELOPING AUTHENTIC CELLULAR MEDICINES

Meeting on the Mediterranean 2022

Our Mission

is to create authentic cellular medicines to reverse devastating diseases.

BlueRock's Core Capabilities

Technology

cell+gene platform

- Manufacture any cell, edit any gene, for any therapeutic purpose



Manufacturing

Integrated CMC capabilities

- Cell production suited for pre-clinical through commercial scale



Products

Pipeline of PSC-based therapeutics

- Leading portfolio of best-in-class cellular medicines



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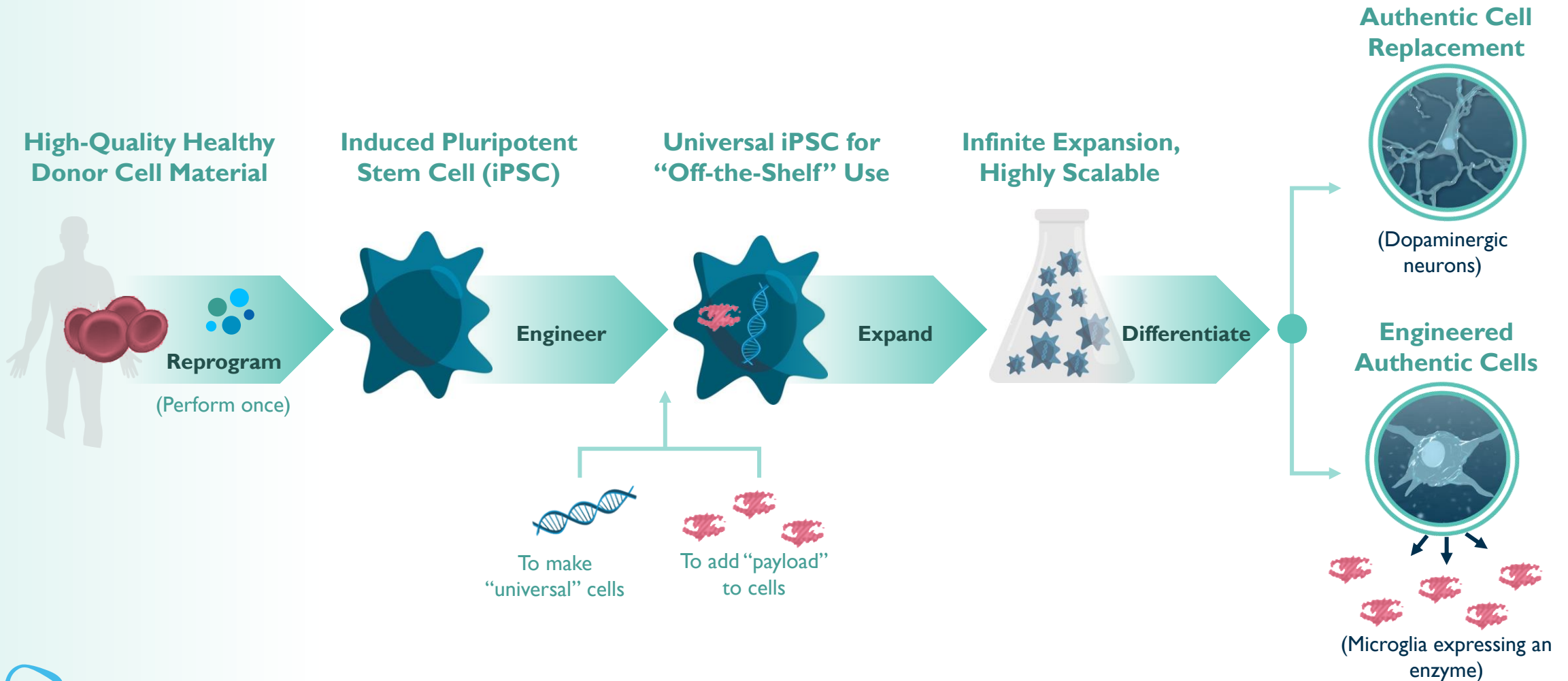
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cell+gene Platform Based on Universal Stem Cells

Enables Both Authentic and Engineered Cellular Medicines



Gene Engineering Capabilities Enable Next-generation Medicines

BlueRock has developed a robust toolbox for iPSC engineering

- Strategies that **increase efficiency** of engineering
- Tools for **always on** and **inducible** cargo expression
- **Functional, durable expression** of secreted payloads, kill switches, and immune evasive molecules
- **GMP workflows**

Key Collaborations



Non-exclusive license to CRISPR gene editing technologies that supports our portfolio in neurology, cardiology, and immunology



SENTI BIO

Combines BlueRock's leading iPSC platform technology and genome biology expertise with Senti Bio's proprietary gene circuit technology platform, including Smart Sensors and Regulator Dials

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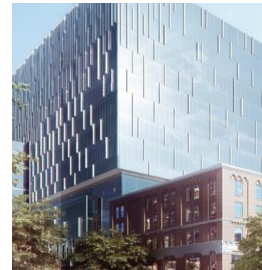
Manufacturing Expertise to Enable Delivery of Cell Therapies

BlueRock's Advantages & Core Competencies:

- End to End expertise, from cell line derivation to device delivery
- Deep understanding of PSC biology to enable manufacturing of new therapeutic modality
- Production at scales relevant to unmet medical needs
- Leveraging CMC expertise to develop key partnerships and set standards for a new industry
- Long-term financial and facilities commitment from BlueRock & Bayer



Toronto, CA
Pilot cGMP Cell Manufacturing Facility



Cambridge, MA
Pilot cGMP for Gene-editing Materials Facility



Berkeley, CA
BRTx/Bayer Commercial Scale Cell Culture Technology Center

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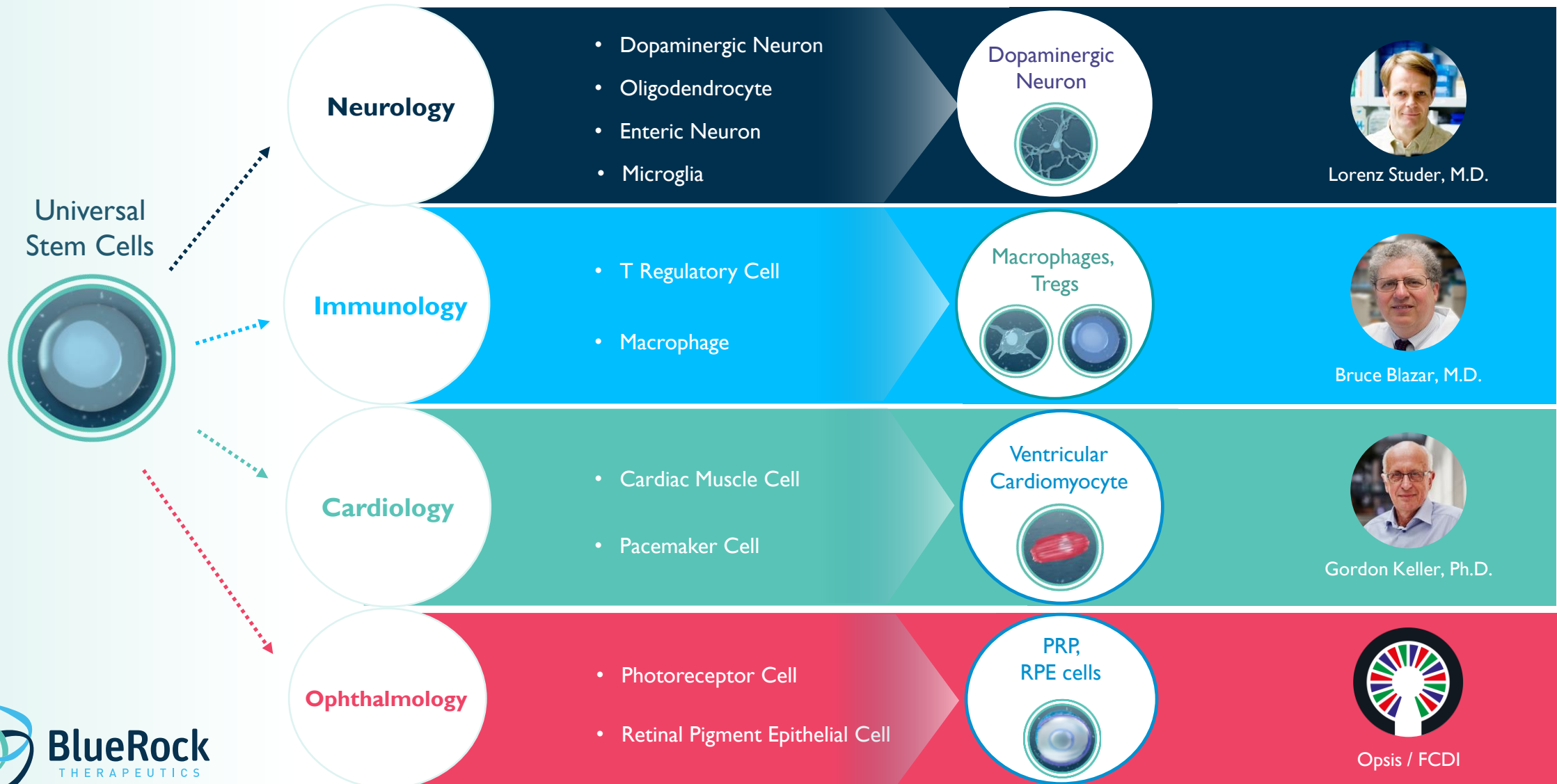


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Pipeline Fueled by World Renowned Collaborators

Unparalleled Cell Design and Differentiation Expertise

Collaborators



Phase I DA01 Parkinson's Trial

Rapid Evaluation of Safety, Tolerability and Efficacy



Memorial Sloan Kettering
Cancer Center



BlueRock
THERAPEUTICS



Weill Cornell
Medicine



University of
California, Irvine



Trial Design

- Multi-center, open label, Phase I trial assessing DA01 authentic cell therapy for Parkinson's Disease



Enrollment Criteria

- Subjects with PD (male/female)
- Patients diagnosed ≥ 3 and ≤ 15 years ago
- Responsive to L-dopa, but inadequate relief of motor symptoms



Objectives

- Safety, tolerability, PET-imaging for cell survival at years 1 & 2
- Preliminary efficacy (motor, non-motor, QoL) at years 1 & 2



Dosing

- Two cohorts - low and high doses
- Immunosuppression up to 12 months following transplantation



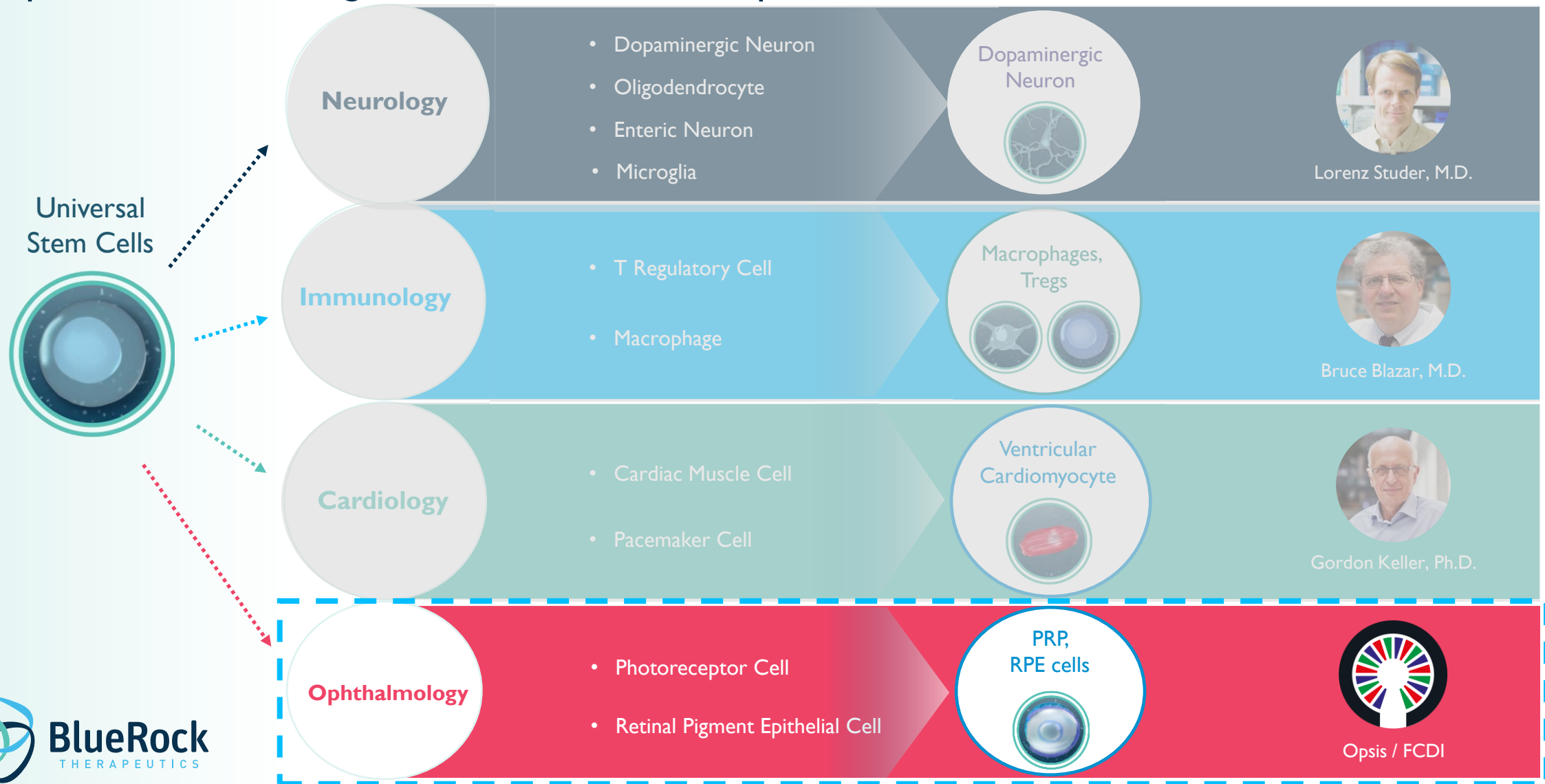
UPDATE

- Closure of Cohort A in Jan. 2022
- First patient dosed in Canada in Jan. 2022

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Partnership With Opsis / FCDI Expands Portfolio into Ophthalmology

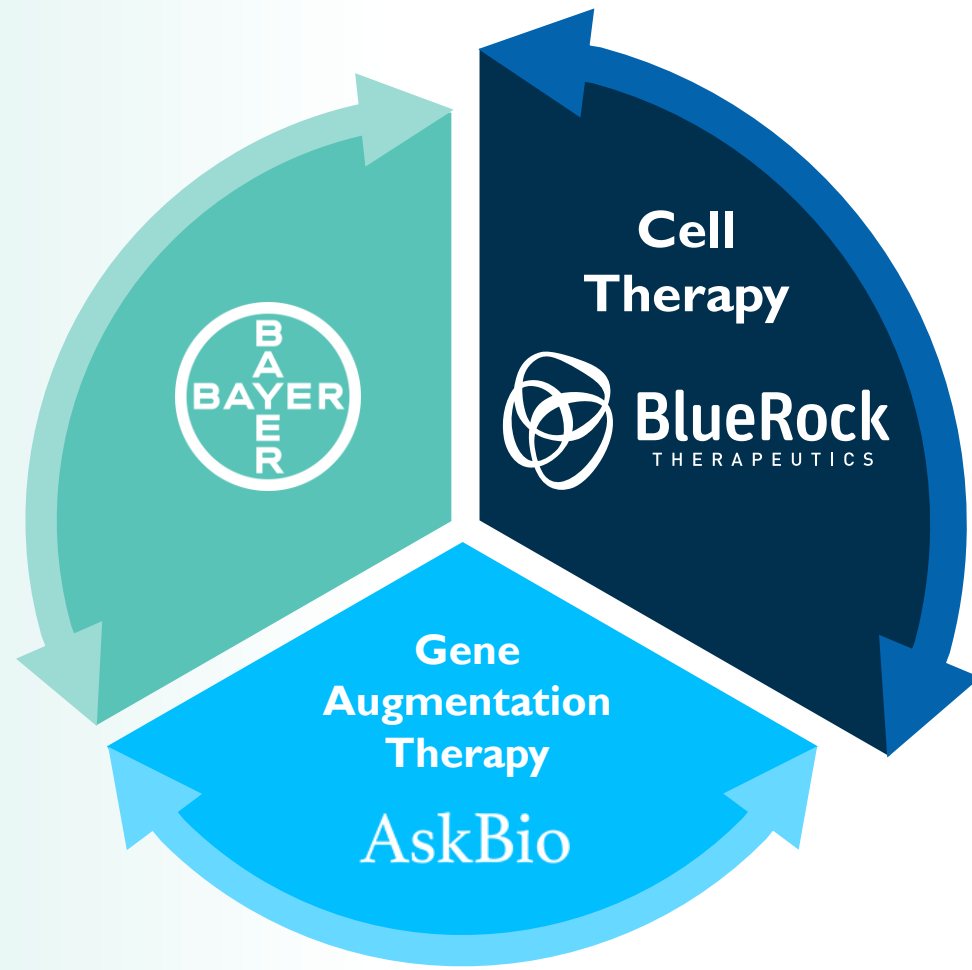
BlueRock Therapeutics, FUJIFILM Cellular Dynamics, and Opsis Therapeutics Enter into a Strategic R&D Alliance to Discover and Develop Cell Therapies for Eye Diseases

CAMBRIDGE, Mass., and MADISON, Wis., May 17, 2021 /PRNewswire/ -- BlueRock Therapeutics



- Three iPSC-based retinal cell therapy programs for **inherited retinal disorders** and in **early and later dry AMD**
 - **OpCT-001**: Photoreceptor Progenitors (PRP) for Inherited Retinal Disorders
 - **OpCT-002**: Combination program (PRP + RPE) for dry AMD
 - **OpCT-003**: Retinal Pigment Epithelial (RPE) cells for dry AMD
- Partnership leverages **each party's strengths** from discovery through commercialization

BlueRock is Foundational to Bayer's Cell and Gene Therapy Platform



- Each of Bayer's C> Platform pillars complement each other, combining the best in Biotech and Pharma know-how
- BlueRock is looking to grow through innovative partnerships that leverage our platform capabilities to develop new medicines for patients

The background is a deep blue gradient. Overlaid on this are numerous thin, white lines that connect small, semi-transparent white dots. These dots and lines are scattered across the frame, with a higher concentration in the upper right quadrant, creating a sense of a digital network or data flow. The overall effect is modern and technological.

Thank You!