# SECOND HALF FEBRUARY 2023 ADVANCED THERAPIES COLLABORATIONS & M&A

REACH OUT TO BRYAN KENNEDY AT BK@PROJECTEVOLUTION.ORG WITH ANY QUESTIONS

## Collaborations

- 03.01.23 - Vertex pays ImmunoGen $15M to use antibody-drug conjugates to improve CRISPR therapy (fiercebiotech)
  - Are antibody-drug conjugates the key to better gene editing treatments? That is a question posed by the latest deal by Vertex, which is paying ImmunoGen $15 million upfront to explore the use of ADCs with its near-approval CRISPR/Cas9 therapy exagamglogene autotemcel (exa-cel). ADCs are best known as cancer drugs. In that context, the pairing of a targeting antibody and a cytotoxic payload can create treatments that wipe out cancer cells without causing unbearable systemic toxicity. Researchers have begun to explore using the same approach to selectively deplete certain cell types to prepare the body for gene therapy and stem cell transplant. Vertex has bet $15 million upfront on that idea, with potentially up to $337 million in option exercise fees and development and commercial milestone payments to follow per target. The deal allows Vertex to use ImmunoGen's ADC technology to discover novel targeted conditioning agents and then obtain a worldwide license for conditioning agents that use ImmunoGen's technology for that target.
- 02.28.23 - Tevard Biosciences announces collaboration with Vertex to develop novel tRNA-based therapies for Duchenne muscular dystrophy (PR)
  - A four-year global research collaboration aimed at creating new tRNA-based therapies for patients with Duchenne muscular dystrophy (DMD) caused by nonsense mutations with options to expand into additional muscular dystrophies and a second indication.The agreement provides access to Tevard's proprietary platforms for discovering and developing tRNA-based therapies. Under its terms, Tevard will receive up-front, option-exercise, and milestone payments, plus royalties on any approved products. Tevard will advance the research and discovery of novel tRNA-based therapies, with all program costs funded by Vertex. Vertex will be responsible for all subsequent development, manufacturing and commercialization.
- 02.23.23 - AbbVie and Capsida Biotherapeutics Expand Strategic Collaboration to Develop Targeted Genetic Medicines for Eye Diseases with High Unmet Need (PR)
  - Under the terms of the expanded agreement, Capsida will receive $70 million, consisting of upfront payments and a potential equity investment. For the three programs, Capsida may be eligible to receive up to $595 million in option fees and research and development milestones, with potential for further commercial milestones. Capsida is also eligible to receive mid-to-high single-digit royalty payments on future product sales. Capsida will lead capsid discovery efforts for all programs using its high throughput AAV engineering platform and will be responsible for process development and early clinical manufacturing. AbbVie will lead innovative therapeutic cargo approaches and be responsible for development and commercialization.
- 02.23.23 - Novartis Drops SCD Gene Therapy Program with Intellia (biospace)
  - Novartis has abandoned its ex vivo sickle cell disease (SCD) program developed using Intellia Therapeutics' CRISPR gene editing platform, according to Intellia's 2022 financial results released Thursday. A representative from Intellia told BioSpace its focus has been "on the development of an in vivo editing approach for the treatment of SCD to avoid the need for bone marrow transplantation." Graphite Bio announced Thursday it will discontinue its gene therapy for SCD following the decision to put the trial on hold after a patient experienced prolonged periods of low blood cell counts from the therapy. CEO Josh Lehrer said continuing nula-cel's development would be an unsound business decision due to the "evolving treatment landscape."
- 02.23.23 - AviadoBio Signs Exclusive License Agreement with Neurgain Technologies for Novel Gene Therapy Spinal Delivery Technology (PR)
  - Subpial delivery technology has the potential to offer precise, efficient, and safer delivery of gene therapies for neurological diseases with spinal cord involvement. Studies in large animal models demonstrate robust biodistribution and potential safety advantages over current spinal cord delivery approaches. Enhances AviadoBio's gene therapy delivery capabilities for neurological diseases affecting the spinal cord. AviadoBio is currently employing subpial delivery as part of its pipeline of ALS gene therapies. Under the agreement, Neurgain has granted AviadoBio an exclusive worldwide license to develop, manufacture and commercialize subpial delivery technology.
- 02.22.23 - Moderna And Life Edit Therapeutics Enter Strategic Collaboration to Accelerate the Development of Novel In Vivo Gene Editing Therapies (PR)
  - Collaboration to combine Moderna's mRNA platform with Life Edit's proprietary gene editing technologies, including base editing capabilities. Multi-target collaboration to advance potentially life-transformative or curative therapies for some of the most challenging genetic diseases. Life Edit to receive upfront cash payment, research, and preclinical funding, and is eligible to receive milestone payments with tiered royalties on global net product sales
- 02.22.23 - TransCode Therapeutics and BRAIN Biotech join forces to develop a CRISPR-derived technology platform for cancer treatment (PR)
  - TransCode Therapeutics, Inc. (Nasdaq: RNAZ), the RNA oncology company committed to more effectively treating cancer using RNA therapeutics, today announced the signing of a non-binding letter of intent and a joint research and development agreement (JDA) with industrial biotechnology and genome editing expert, BRAIN Biotech AG. The objective of the JDA is to co-develop a platform technology that combines a Class 2 CRISPR nuclease, the cell-killing G-dase E, developed by BRAIN Biotech's Akribion Genomics unit with TransCode's TTX nucleic acid delivery platform for the treatment of cancer.
- 02.22.23 - Eterna Therapeutics Enters Into Option and License Agreement with Lineage Cell Therapeutics to Develop Hypoimmune Pluripotent Cell Lines for Multiple Neurology Indications (PR)
  - Eterna, a preclinical-stage biotechnology company committed to realizing the potential of mRNA cell engineering for neurology indications. Eterna is the exclusive licensee of the key intellectual property underlying this collaboration. Under the Agreement, Eterna plans to conduct certain gene-editing activities and provide materials to Lineage for evaluation. The Agreement provides Lineage an option to obtain an exclusive license to utilize and sublicense the novel gene-edited cell lines for preclinical, clinical, and commercial purposes in the field of CNS diseases. A feature of the starting cell line is the targeted deletion of the beta 2 microglobulin (B2M)-gene, which is designed to reduce the immunogenicity of product candidates derived from the lines by inhibiting rejection by CD8+ T cells.

- 02.16.23 - Indee Labs Partners with GenScript to Develop Direct DNA Knock-in Kit for Large Transgene Inserts (PR)
  - Indee Labs, the developer of Hydropore™ for non-viral intracellular delivery, is partnering with GenScript USA Inc., the world's leading provider of life-science research tools and services, to deliver GenScript's non-viral DNA payloads into cells at greater efficiency, and to enable the integration of longer DNA inserts than those allowed by traditional viral methods. As part of the collaboration, GenScript is providing a variety of single-strand and double-strand (ssDNA and dsDNA) constructs for direct DNA inserts, while the team at Indee Labs is optimizing the Hydropore protocol and evaluating functional differences between T cells processed with Hydropore and electroporation. Looking ahead, the companies will work on co-marketing a complete non-viral solution to the gene and cell therapy community.
- 02.16.23 - Akamis Bio, Parker Institute for Cancer Immunotherapy, and Cancer Research Institute Announce Expanded Partnership to Advance Novel Treatments for Pancreatic Cancer.
  - As part of the Akamis Bio, PICI, and CRI partnership, NG-350A, an immuno-stimulatory tumor gene therapy driving intratumoral expression of a CD40 agonist monoclonal antibody, will be evaluated in combination with standard-of-care chemotherapy and the CTLA-4 inhibitor ipilimumab (YERVOY®). The NG-350A combination therapy will be part of cohort C in REVOLUTION, a platform clinical study investigating novel therapeutic combinations for the treatment of previously untreated metastatic pancreatic cancer. In 2018, Akamis Bio initiated a pre-clinical partnership with PICI, a network representing the largest concentration of immuno-oncology expertise in the world, with a focus on research to investigate the use of the company's T-SIGn® therapeutics for the treatment of solid tumors. Akamis Bio's relationship with PICI, which includes a financial investment by PICI in the company, has now expanded to include a clinical collaboration thanks to the generous financial and operational support of CRI for the inclusion of NG-350A in the REVOLUTION study. Akamis Bio's clinical-stage NG-350A tumor gene therapy to be included as a novel combination therapeutic agent in a new cohort of the REVOLUTION platform study
- 02.14.23 - Gritstone bio and the National Cancer Institute (NCI) Establish a Clinical Trial Agreement to Evaluate a Neoantigen Cell Therapy-Vaccine Combination (PR)
  - NCI will lead the Phase 1 study using Gritstone's proprietary "off the shelf" vaccine technology for mutant KRAS solid tumors. Under the terms of the agreement, NCI will identify patients with metastatic cancer that are eligible for adoptive cell transfer based on the presence of a G12V or G12D KRAS mutation (KRASmut). Gritstone will provide the SLATE-KRAS vaccine as requested by NCI for the trial.

## M&A

- 02.22.23 - Kite Completes Acquisition of Tmunity (PR)
  - The acquisition of Tmunity complements Kite's existing in-house cell therapy research capabilities by adding additional pipeline assets, platform capabilities, and a strategic research and licensing agreement with the University of Pennsylvania (Penn). It will provide Kite with access to pre-clinical and clinical programs, including an 'armored' CAR T technology platform, which potentially could be applied to a variety of CAR T's to enhance anti-tumor activity, as well as rapid manufacturing processes. In addition, as part of the acquisition, the Tmunity founders, who remain in their roles at Penn, will also provide consulting services to Kite as senior scientific advisors.
- 02.16.23 - By merging with struggling Erytech, Pherecydes spies opportunity to boost AMR strategy (fiercebiotech)
  - In the year and a half since Erytech Pharma was blindsided by a phase 3 fail that forced a pivot from pancreatic cancer to leukemia, the company has been feeling out strategic options. Now, fellow French biotech Pherecydes has spotted an opportunity to take advantage of Erytech's money, expertise and U.S. footprint via a merger. The combined company will maintain Pherecydes' focus on extended phage therapies—natural bacteria-killing viruses—to combat antimicrobial resistance (AMR). The new, as yet unnamed entity will have around 41 million euros ($35.6 million) in the bank that could fund both current and new clinical programs into the third quarter of 2024. Pherecydes already has plans for two new midstage trials in the U.S. One study in patients with endocarditis due to S. aureus is expected to launch in the middle of the year, with a trial in complex urinary tract infections due to E. coli penciled in for the first quarter of 2024.