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January 2023 Advanced Therapies Collaborations & M&A

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

- 01.31.23 - uniQure and Apic Bio enter into a global licensing agreement for APB-102, a clinical-stage gene therapy for patients with ALS caused by mutations in SOD1 (PR)
  - APB-102 further strengthens uniQure's pipeline of innovative gene therapies to treat neurological disorders and miRNA-based gene silencing programs. APB-102 and uniQure's c9orf72-ALS program have the potential to address most inherited forms of ALS. UniQure plans to initiate a Phase I/II trial of APB-102 in the second half of 2023. The clinical development of APB-102 is based on nearly 30 years of research demonstrating the link between the SOD1 gene mutation and ALS. Under the terms of the agreement, uniQure will make an initial cash-payment of $10M, and up to $45M in milestone payments upon specific regulatory approvals (FDA, EMA) and pre-specified annual net sales, based on tiered royalty.
- 01.27.23 - Spark Therapeutics Enters Into Strategic Collaboration with Neurochase for Use of Proprietary Delivery Technology for CNS Disorders (PR)
  - Neurochase is developing a proprietary drug delivery system and this collaboration enables Spark to develop selected gene therapies for CNS disorders using the Neurochase technology, which aims to improve targeted delivery of AAV gene therapy to neural structures using the method of Convection Enhanced Delivery (CED).
- 01.25.23 - GenKOre Announces Collaboration with a US-based Company on In vivo Gene-editing Therapy (PR)
  - Strategic research collaboration with a US-based biopharma company for the development of in vivo gene editing therapies. This collaboration will utilize GenKOre's proprietary CRISPR-Cas platform, TaRGET (Tiny nuclease, augment RNA-based Genome Editing Technology). The TaRGET platform is distinct from the most popular genome editing technology, CRISPR-Cas9, in that the whole editing module can be delivered with a single AAV vector. Based on their TaRGET platform, GenKOre has developed different modalities of editing tools including TaRGET-CUT, TaRGET-Adenine Base Editing (ABE), TaRGET-AI (Gene Activation and Inhibition system). and TaRGET-FREE (Gene knock-in) .
- 01.24.23 - ATUM Announces Leap-In Transposase® Licensing Agreement with Codiak Biosciences to Support Manufacturing of Exosome-Based Therapeutics (PR)
  - Under the terms of the agreement, ATUM will provide Codiak access to its Leap-In Transposase® technology to generate mammalian cells expressing exosomes engineered to deliver therapeutic payloads. The Leap-In technology is an integrated solution combining proprietary re-coding algorithms, unique genetic vector elements as well as a transposon-based mechanism for efficient insertion of genetically stable elements into the genome.
- 01.11.23 - Arbor Biotechnologies Announces Expanded Strategic Partnership with Vertex, Now Extending to Precision Gene Editing Using Reverse Transcriptase (PR)
  - Arbor announced the expansion of an existing strategic collaboration covering in vivo genetic medicines by which Vertex Pharmaceuticals Incorporated (Vertex) will receive rights to Arbor's novel precision editing technology for up to three diseases. Under the terms of the agreement, Arbor is eligible to receive payments based upon the successful achievement of specified research, development, regulatory and commercial milestones. In addition, Vertex will pay tiered royalties on future net sales of any products that may result from this collaboration. This marks a significant expansion of the relationship between the two companies, who first partnered on gene-editing therapies in 2018 and entered into a new relationship in 2021 around next-generation cell therapy approaches in diabetes and hemoglobinopathies, among other diseases.
- 01.11.23 - Solid Biosciences and Phlox Therapeutics Announce Strategic Research Collaboration Focused on Accelerating the Development of New Therapies for Rare Cardiac Diseases (PR)
  - Research collaboration to target severe form of genetic dilated cardiomyopathy. Collaboration strengthens Solid Biosciences' scientific capabilities and commercial potential in cardiac therapy. Collaboration allows Phlox Therapeutics to leverage Solid Biosciences' vector biology and manufacturing capacities to deliver its RNA therapies to the heart. The strategic collaboration will integrate Solid Biosciences' vector biology, manufacturing capabilities and drug development experience with Phlox's deep expertise in genetic cardiomyopathies and RNA therapeutics. The companies will collaborate to develop novel precision genetic medicines for this form of DCM.
- 01.11.23 - Myrtelle and rAAVen Therapeutics to Develop Novel Gene Therapy Vectors (PR)
  - The partnership will leverage rAAVen's expertise in modifying established viral vectors for the design and production of new viral vectors as potential precision treatments for a broad spectrum of diseases in combination with Myrtelle's expertise in CNS gene therapy research and development. rAAVen will utilize its unique platform for AAV development that combines state-of-the-art methodologies within cloning, viral vector production and next generation sequencing. Myrtelle will test the vectors in a range of myelin-based disorders and, if successful, pursue further development. Myrtelle will own the vector compositions and retain exclusive worldwide rights to commercialize the resulting gene therapies. In return, rAAVen will receive milestone and sales-based royalty payments.
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- 01.10.23 - AbbVie and Anima Biotech Announce Collaboration for the Discovery and Development of mRNA Biology Modulators (PR)
  - Under the terms of the agreement, Anima will receive an upfront payment of $42 million and may be eligible to receive up to $540 million in option fees and research and development milestones in the aggregate across the three targets, with potential for further commercial milestones as well as tiered royalties on net sales. AbbVie has an option to expand the collaboration with up to three additional targets under the same terms as the initial collaboration, which may increase the potential value of the collaboration.
- 01.09.23 - Selecta Biosciences and Astellas Announce Exclusive Licensing and Development Agreement for Xork IgG Protease (PR)
  - an exclusive licensing and development agreement for IdeXork (Xork). Xork is being studied as a potential next generation immunoglobulin G (IgG) protease that will be developed by Astellas for use with AT845, an investigational, adeno-associated virus (AAV)-based treatment for Late-Onset Pompe disease (LOPD) in adults. Under the terms of the agreement, Selecta will receive a $10M upfront payment and is eligible to receive up to $340M for certain additional development and commercial milestones plus royalties on any potential commercial sales where Xork is used as a pre-treatment for AT845. Selecta is responsible for the development and manufacturing of Xork and will maintain the rights for the development of additional indications beyond Pompe disease. Astellas would have the sole and exclusive right to commercialize Xork for use in Pompe disease with an Astellas gene therapy investigational or authorized product, with a current focus on AT845.
- 01.09.23 - Boehringer Ingelheim and 3T Biosciences Join Forces to Develop Next-Generation Cancer Immunotherapies (PR)
  - Under the agreement, Boehringer Ingelheim will provide patient-derived TCR data to fuel 3T's target discovery efforts to identify antigens using its 3T TRACE discovery platform. 3T will receive an upfront payment and research and development support, and is eligible for discovery, preclinical, clinical, regulatory, and commercial milestones totaling $268 million in addition to royalties on future Boehringer Ingelheim product sales. Boehringer Ingelheim is eligible to receive royalties on future product sales by 3T Biosciences arising from the agreement.
- 01.09.23 - Neurocrine Biosciences and Voyager Therapeutics Enter Strategic Collaboration for Development and Commercialization of Voyager's GBA1 Program and Other Next-Generation Gene Therapies for Neurological Diseases (PR)
  - Voyager to receive up-front consideration of $175 million including a $39 million equity investment, up to $1.5 billion in potential development milestones, additional potential commercial milestones, tiered royalties on net sales, program funding, and an option to elect 50/50 cost- and profit-sharing in the U.S. for the GBA1 program following Phase 1 readout. Neurocrine to receive worldwide rights to Voyager's GBA1 gene therapy program for Parkinson's disease and other GBA1-mediated diseases and three gene therapy programs directed to rare CNS targets, each enabled by Voyager's next-generation TRACERTM capsids, as well as additional equity in Voyager. Jude Onyia, Ph.D., Chief Scientific Officer at Neurocrine Biosciences, will join Voyager's Board of Directors.
- 01.09.23 - Autolus Therapeutics Announces Collaboration with Cabaletta Bio for Use of Autolus' Safety Switch System in Cell Therapies for Autoimmune Disease (PR)
  - Under the terms of the agreement, Autolus will receive an upfront payment for non-exclusive access to the RQR8 safety switch for use in Cabaletta's CD19-CAR T cell therapy program for the treatment of autoimmune disease, with the potential for near term option exercise fees and development and regulatory milestone payments. In addition, Autolus is entitled to receive royalties on net sales of all Cabaletta cell therapy products that incorporate the RQR8 safety switch.
- 01.04.23 - Capsida Biotherapeutics Announces Strategic Collaboration with Prevail, a Wholly Owned Subsidiary of Lilly, to Develop Non-Invasive Gene Therapies for CNS Diseases (PR)
  - multi-year strategic collaboration with Prevail Therapeutics, a wholly owned subsidiary of Eli Lilly and Company , to develop transformative genetic medicines for serious diseases. As part of the collaboration, Prevail will leverage Capsida's novel AAV engineering platform to identify and advance clinically translatable capsids paired with Prevail's cargo to develop best-in-class, IV-administered gene therapies directed to specified targets known to cause serious diseases that affect the central nervous system (CNS). Delivering AAV gene therapy systemically to target the CNS, while limiting exposure to non-target organs (such as the liver), has been a significant challenge in the gene therapy field. Capsida has developed a high throughput platform to biologically screen and identify engineered AAV capsids that target specific tissues, such as the brain, and limit transduction of tissues and cell types that are not relevant to a given disease. This platform has the potential to improve the efficacy and safety of systemically administered AAV gene therapies and deliver on the promise of gene therapy for patients that are living with diseases that are difficult to treat with existing approaches.
- 01.03.23 - MaxCyte Signs Strategic Platform License with Catamaran Bio to Support its CAR-NK Cell Therapy Programs (PR)
  - MaxCyte, a leading commercial cell-engineering company providing enabling platform technologies to advance innovative cell-based research, as well as next-generation cell therapeutic discovery, development and commercialization, today announced the signing of a strategic platform license (SPL) with Catamaran Bio, Inc., a biotechnology company developing novel, off the-shelf chimeric antigen receptor (CAR)-NK cell therapies to treat a broad range of cancers, with a primary focus on solid tumors. Under the terms of the agreement, Catamaran obtains non-exclusive clinical and commercial rights to use MaxCyte's Flow Electroporation® technology and ExPERT™ platform. In return, MaxCyte will receive platform licensing fees and program-related revenue.

M&A Updates

- 01.30.23 - Kite and Arcellx Close Agreement to Co-develop and Co-commercialize Late-stage Clinical CART-ddBCMA in Multiple Myeloma (PR)
  - previously announced global strategic collaboration to co-develop and co-commercialize Arcellx's lead late-stage product candidate, CART-ddBCMA, for the treatment of patients with relapsed or refractory multiple myeloma. Multiple myeloma is an incurable disease for most patients and the need remains for effective, safe and broadly accessible therapies. Currently being investigated in a Phase 2 pivotal trial, CART-ddBCMA is Arcellx's T-cell therapy utilizing the company's novel synthetic binder, the D-Domain. Kite and Arcellx will jointly advance and commercialize the CART-ddBCMA asset in the U.S., and Kite will commercialize the product outside the U.S.

- 01.27.23 - BridgeBio company sells off rare disease candidate to Galderma partner (endpts)
  - Israeli biotech Sol-Gel Technologies announced Friday that it got its hands on a rare disease drug candidate from PellePharm for almost $75 million, amid claims that the drug has the potential to reach a $300 million market. The biotech said that it will be paying PellePharm $4.7 million upfront with an additional $70 million in development, NDA and commercial milestones, plus single-digit royalties. The deal is expected to close on Jan. 30.PellePharm is a BridgeBio company that has been quiet in recent years, with its last press release having been published in October of 2020. Back in 2018, Leo Pharma took a minority stake in PellePharm, agreeing to provide R&D support to the company that was planning on testing patidegib in a Phase III.
- 01.25.23 - KSQ Therapeutics sells early-stage targeted therapies to Japan's Ono (Endpts)
  - As it moves deeper into a Phase I trial of its lead program, KSQ Therapeutics is selling a slate of earlier-stage oncology programs in exchange for some cash. Japan's Ono Pharmaceutical is paying a "double-digit million upfront" to secure multiple research-stage drugs designed to attack cancer by targeting DNA damage response pathways. KSQ identified those programs — all with first-in-class potential, according to the companies — using its CRISPR screening platform.
- 01.16.23 - Acquisition of Neogene Therapeutics completed
  - AstraZeneca has completed the acquisition of Neogene Therapeutics Inc. (Neogene), a global clinical-stage biotechnology company pioneering the discovery, development and manufacturing of next-generation T-cell receptor therapies (TCR-Ts). Neogene will operate as a wholly owned subsidiary of AstraZeneca, with operations in Amsterdam, the Netherlands and California, US. Financial considerations. AstraZeneca has acquired all outstanding equity of Neogene in exchange for an initial payment of $200m. Under the terms of the agreement, AstraZeneca will pay up to $120m in additional contingent milestone-based and non-contingent consideration
- 01.19.23 - Editas, in next restructuring step, to sell cell therapy work to Shoreline (biopharmadive)
  - Editas will sell its cancer cell therapy work to privately held Shoreline Biosciences as part of a company-wide restructuring that's already led to layoffs and cutbacks in spending. The deal, announced by the companies Thursday, has Shoreline acquiring Editas' natural killer, or NK, cell therapy franchise, including a preclinical-stage treatment for solid tumors. Shoreline will also license Editas' gene editing technology for use in modifying NK cells and macrophages, another type of white blood cell. Shoreline will make an undisclosed upfront payment to Editas, and has promised additional payments should its work reach certain development and commercial milestones. Shares in Editas fell by nearly 4% Thursday morning on news of the deal.
- 01.19.23 - Inscripta Acquires Infinome Biosciences, Sestina Bio (Genomeweb)
  - Genome editing firm Inscripta said on Wednesday that it has acquired two synthetic biology firms, Infinome Biosciences and Sestina Bio, for an undisclosed amount. The moves suggest the firm has pivoted to biomanufacturing, away from genome editing instrumentation. Infinome, based in Boulder, Colorado, has developed a platform for rapidly engineering microbial strains, dubbed GenoScaler. Inscripta invested in Infinome in March 2021, calling it the firm's "first subsidiary" at the time. Sestina Bio, located in Pleasanton, California, has developed an approach for building and identifying strains that can withstand scale-up to the levels needed for commercial biomanufacturing. In October, the firms announced that Sestina Bio had used Inscripta's Onyx genome editing platform to develop a microbial strain that can produce bakuchiol, a potential skincare ingredient.

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PROJECT EVOLUTION

Project Evolution is an innovative, end-to-end, HUMAN CAPITAL platform that enables leading Life Scientific organizations to identify and secure the talent required to drive innovation. Our collaborative platform is powered by the industry-leading capabilities of Berke Executive Search and GTS Scientific. The highly-successful Joint Venture bridge Executive and Contingent placement and enable organizations of all sizes the ability to secure highly specialized talent quickly and efficiently.

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