Selecta Biosciences and AskBio Receive FDA Rare Pediatric Disease Designation for their Gene Therapy for Methylmalonic Acidemia

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WATERTOWN, Mass. and RESEARCH TRIANGLE PARK, N.C., Oct. 20, 2020 (GLOBE NEWSWIRE) -- **Selecta Biosciences, Inc. (NASDAQ: SELB)** and Asklepios BioPharmaceutical, Inc. (AskBio), today announced the U.S. Food and Drug Administration (FDA) has granted Rare Pediatric Disease Designation to MMA-101 for the treatment of isolated methylmalonic acidemia (MMA) due to methylmalonyl-CoA mutase (MMUT) gene mutations. The FDA grants Rare Pediatric Disease Designation to incentivize development of new treatments for serious and life-threatening diseases that primarily affect children ages 18 years or younger with fewer than 200,000 people affected in the U.S. The Rare Pediatric Disease designation program allows for a Sponsor who receives an approval for a product to potentially qualify for a voucher that can be redeemed to receive a priority review of a subsequent marketing application for a different product.

“This Rare Pediatric Disease designation from the FDA highlights the significant unmet medical need that Selecta and AskBio are seeking to address with MMA-101 for this rare metabolic disorder,” said Carsten Brunn, Ph.D., chief executive officer of Selecta Biosciences. “When used with AAV gene therapy vectors, Selecta’s ImmTOR aims to inhibit the immune response to the AAV vector, potentially allowing re-dosing of gene therapies. Ongoing clinical programs will focus on evaluating product candidate performance in patients who may have been underdosed or those who may lose transgene expression over time. We’re honored to receive this recognition and look forward to advancing this program in hopes of helping young patients affected by MMA and their families.”

“MMA is a serious and potentially life-threatening inherited metabolic disorder that presents in patients from newborns to adulthood,” said Sheila Mikhail, J.D., CEO and co-founder of AskBio. “AskBio is committed to delivering transformative genetic medicines for rare diseases like this one, and the Rare Pediatric Disease designation helps us continue development of MMA-101.”

AskBio and Selecta expect to initiate a Phase 1 clinical trial of MMA-101 and ImmTOR for patients with MMA in 1H 2021.

About Methylmalonic Acidemia

Methylmalonic Acidemia (MMA) is a rare monogenic disorder in which the body cannot break down certain proteins and fats. This metabolic disease may lead to hyperammonemia and is associated with long-term complications including feeding problems, intellectual disability, chronic kidney disease and inflammation of the pancreas. Symptoms of MMA usually appear in early infancy and vary from mild to life-threatening. Without treatment, this disorder can lead to coma and in some cases death.

About Selecta Biosciences, Inc.

Selecta Biosciences, Inc. (NASDAQ: SELB) is leveraging its clinically validated ImmTOR™ platform to develop tolerogenic therapies that selectively mitigate unwanted immune responses. With a proven ability to induce tolerance to highly immunogenic proteins, ImmTOR has the potential to amplify the efficacy of biologic therapies, including redosing of life-saving gene therapies, as well as restore the body’s natural self-tolerance in autoimmune diseases. The company’s first program aimed at addressing immunogenicity to AAV gene therapies is expected to enter clinical trials in early 2021 in partnership with AskBio for the treatment of methylmalonic acidemia (MMA), a rare metabolic disorder. A wholly-owned program focused on addressing IgA nephropathy driven by ImmTOR and a therapeutic enzyme is also in development among additional product candidates. Selecta recently licensed its Phase 3 clinical product candidate, SEL-212, in chronic refractory gout to Sobi. For more information, please visit www.selectabio.com.

About AskBio

Founded in 2001, Asklepios BioPharmaceutical, Inc. (AskBio) is a privately held, fully integrated AAV gene therapy company dedicated to developing life-saving medicines that cure genetic diseases. Its pipeline includes clinical-stage programs in Pompe disease and congestive heart failure and a diverse preclinical portfolio of therapeutics targeting neuromuscular, CNS and other diseases, as well as out-licensed clinical indications for hemophilia (Chatham Therapeutics, acquired

therapeutics targeting neuromuscular, CNS and other diseases, as well as out-licensed clinical indications for hemophilia (Chatham Therapeutics, acquired by Takeda) and Duchenne muscular dystrophy (Bamboo Therapeutics, acquired by Pfizer). AskBio's gene therapy platform includes Pro10™, an industry-leading proprietary cell line manufacturing process, and an extensive AAV capsid and promoter library. With global headquarters in Research Triangle Park, North Carolina, and European headquarters in Edinburgh, UK, the company has generated hundreds of proprietary third generation AAV capsids and promoters, several of which have entered clinical testing. An early innovator in the space, the company holds more than 500 patents in areas such as AAV production and chimeric and self-complementary capsids.

Selecta Forward-Looking Statements

Any statements in this press release about the future expectations, plans and prospects of Selecta Biosciences, Inc. ("the company"), including without limitation, statements regarding the unique proprietary technology platform of the company, and the unique proprietary platform of its partners, the potential of ImmTOR to enable re-dosing of AAV gene therapy, the potential treatment applications of product candidates utilizing the ImmTOR platform in areas such as gene therapy and MMA, the company's plans to initiate a clinical trial for a product candidate to treat MMA, the ability of the company and AskBio to develop gene therapy products using ImmTOR and AskBio's technology, any development plans of the company and AskBio have for product candidates to treat serious and life-threatening diseases and the intention to seek regulatory approval thereof, the novelty of treatment paradigms that the Company is able to develop, the potential of any therapies developed by the company and AskBio to fulfill unmet medical needs, the company's plan to apply its ImmTOR technology platform to a range of biologics for rare and orphan genetic diseases, the potential of the company's intellectual property to enable repeat administration in gene therapy product candidates and products, the ability to re-dose patients and the potential of ImmTOR to allow for re-dosing, the potential to safely re-dose AAV, the ability to restore transgene expression, the potential of the ImmTOR technology platform generally and the company's ability to grow its strategic partnerships, and other statements containing the words "anticipate," "believe," "continue," "could," "estimate," "expect," "hypothesize," "intend," "may," "plan," "potential," "predict," "project," "should," "target," "would," and similar expressions, constitute forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including, but not limited to, the following: the uncertainties inherent in the initiation, completion and cost of clinical trials including proof of concept trials, including the uncertain outcomes, the availability and timing of data from ongoing and future clinical trials and the results of such trials, whether preliminary results from a particular clinical trial will be predictive of the final results of that trial or whether results of early clinical trials will be indicative of the results of later clinical trials, the unproven approach of the company's ImmTOR technology, potential delays in enrollment of patients, undesirable side effects of the company's product candidates, its reliance on third parties to manufacture its product candidates and to conduct its clinical trials, the company's inability to maintain its existing or future collaborations, licenses or contractual relationships, its inability to protect its proprietary technology and intellectual property, potential delays in regulatory approvals, the availability of funding sufficient for its foreseeable and unforeseeable operating expenses and capital expenditure requirements, the company's recurring losses from operations and negative cash flows from operations raise substantial doubt regarding its ability to continue as a going concern, substantial fluctuation in the price of its common stock, and other important factors discussed in the "Risk Factors" section of the company's most recent Quarterly Report on Form 10-Q, and in other filings that the company makes with the Securities and Exchange Commission. In addition, any forward-looking statements included in this press release represent the company's views only as of the date of its publication and should not be relied upon as representing its views as of any subsequent date. The company specifically disclaims any intention to update any forward-looking statements included in this press release.

AskBio Forward-Looking Statements

This press release contains "forward-looking statements" regarding AskBio. Any statements contained in this press release that are not statements of historical fact may be deemed to be forward-looking statements. Words such as "believes," "anticipates," "plans," "expects," "will," "intends," "potential," "possible" and similar expressions are intended to identify forward-looking statements. These forward-looking statements include statements regarding MMA-101, including the potential timing of the Phase 1 clinical trial for patients with MMA, AskBio's pipeline of development candidates; AskBio's goal of developing life-saving medicines aimed at curing genetic diseases; the potential benefits of AskBio's development candidates to patients.

These forward-looking statements involve risks and uncertainties, many of which are beyond AskBio's control. Known risks include, among others: AskBio may not be able to execute on its business plans and goals, including meeting its expected or planned regulatory milestones and timelines, clinical development plans and bringing its product candidates to market, due to a variety of reasons, including the ongoing COVID-19 pandemic, possible limitations of company financial and other resources, manufacturing limitations that may not be anticipated or resolved in a timely manner, potential disagreements or other issues with our third-party collaborators and partners, and regulatory, court or agency feedback or decisions, such as feedback and decisions from the United States Food and Drug Administration or the United States Patent and Trademark Office.

Any of the foregoing risks could materially and adversely affect AskBio's business and results of operations. You should not place undue reliance on the forward-looking statements contained in this press release. AskBio does not undertake any obligation to publicly update its forward-looking statements based on events or

looking statements contained in this press release. AskBio does not undertake any obligation to publicly update its forward-looking statements based on events or circumstances after the date hereof.

For more information please contact:

Selecta:
For Investors:
Lee M. Stern
Solebury Trout
+1-646-378-2922
lstern@soleburytrout.com

For Media:
Meredith Sosulski, Ph.D.
LifeSci Communications, LLC
+1-929-469-3851
msosulski@lifescicomms.com

AskBio:
Robin Fastenau
Vice President, Communications
+1-984-275-2705
rfastenau@askbio.com

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