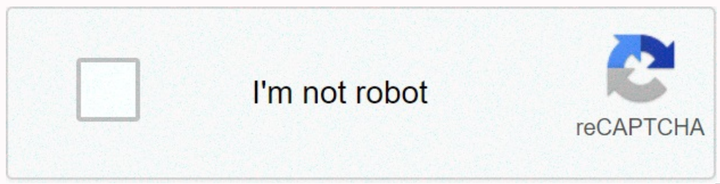

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Top 10 orphan drugs

February is Rare Diseases Day, February 29 - A month-long celebration focused on the suffering and victory of patients and their families suffering from rare diseases that culminate in International Rare Disease Day. In recent years, the rapidly developing field of cell and gene therapy, along with nearly 800 ongoing rare disease-focused clinical development projects, It caused a boom. The biotechnology industry is seeing a new window to improve the lives of patients with rare diseases and potentially treat rare diseases that affect one in 10 Americans and about 400 million people worldwide. Biohealth Metropolitan (BHCR) is a leader in the rare disease market, with U.S. gene technology, REGENXBIO, Cerecor, Aslepion Pharmaceuticals, Radiant and others entering the pipeline with rare disease treatments and treatments. The region's robust rare disease sector also includes many companies that support the research and development of rare diseases such as Yeva Indevitology, GeneDx, Aperiomix and Hemoscher. In short, the portfolio and growth support ecosystem of rare disease companies make BHCR a global hub for rare disease R&D. Maryland already houses the largest number of centers of excellence for communities with rare diseases, and the world travels to us. Roughly speaking, 50% of rare disease patients are children who will never see their 10th birthday and we need these numbers to change. Currently, Maryland is on the radar to become the next top biotech state and has no time to wait as the mother of two rare disease patients, said Lydia Seiders, an independent global strategic adviser to the Rare Diseases Community and national ambassador for the Rare Disorders (NORD) Maryland Rare Action Network. All biotechnology investing in and pursuing more effective rare disease treatments and potential treatments understands the urgency of Seiders. Here's a look at some of BHCR's major companies and what to expect from the pipeline's rare disease program in 2020. Here's a snapshot of five major companies and a pipeline of rare diseases: American Gin Technologies (AGT), Rockville, MD American Gin Technologies is a cell and gene therapy company that leverages its proprietary lenstivirus platform to develop potential treatments for liver cancer, phenylketoñoria (PKU), HIV and genetic diseases. AGT's new lens virus platform has allows companies to develop, test and bank thousands of lentivirus vectors, which can be matched to develop treatments for orphan/rare diseases such as PKU, as well as new targeted diseases, including diseases with large patient populations. PKU is a genetic and mono-genetic disease that causes an over-accumulation of the amino acid phenylalanine (Phe). Toxic levels can be reached in the blood without strict dietary control. Children born with PKU (PKU occurs in about 1 in every 13,500 births) can suffer brain and organ defects if not placed on a special diet, and adults may suffer neuro-mental disorders, including poor behavioral inhibition and limited executive function with a high risk of self-harm. AGT is committed to curing PKU through its proprietary lentivirus platform. The company received the FDA orphan drug designation in 2018 and is conducting PKU treatments towards the clinic. REGENXBIO, Rockville, MD REGENXBIO is a leading clinical stage biotechnology company that develops gene therapy for retinal, neurodegenerative and metabolic diseases. The company's proprietary NAV platform was invented to discover the next generation of adeno-related virus (AAV) vectors to drive the discovery of new therapies in a wide range of disease areas, including rare/orphan diseases. REGENXBIO has exclusive rights to more than 100 new AAV vectors and more than 100 patents and patent applications worldwide. REGENXBIO currently has 13 clinical stage products and is partnering through 20 partnerships. One of their unauthorized programs provided an important milestone for the company and the rare disease community. FDA approval of AVALGENSMA (R) of Avexis, developed on REGENXBIO's NAV (R) technology platform, became the first treatment for spinal muscular atrophy (SMA) and a monumental victory in the use of gene therapy in the field of rare diseases. SMA is a deadly childhood neuro-muscle disease caused by mutations in a single gene. The company's pipeline includes candidates for several rare diseases. RGX-121 is a product candidate for Hunter Syndrome (Mysthetic Pharidosis Type II), a rare X- linked concave disease that causes developmental delays and developmental regression in children. In December 2019, the company announced that it had successfully developed clinical safety and efficacy tests into a second cohort of patients at higher dosage levels. Interim data for the second cohot should be released by about mid-2020. THE FIRST COHOST OF DOSING PATIENTS HAS RESULTED IN POSITIVE SAFETY AND EFFICACY OUTCOMES, INCLUDING MEANINGFUL AND CONTINUOUS REDUCTIONS IN HEPARAN SULFATE, SUGGESTING THAT GENE THERAPY CAN POTENTIALLY RESTORE INTRA-CELL ACTIVITY OF I2S ENZYMES, AS WELL AS EARLY SIGNS OF OBSERVED NEURO-COGNITIVE STABILITY, SAID STEVE PAKOLA, M.D., REGENXBIO'S CHIEF MEDICAL OFFICER. RGX 501 is a treatment being developed for homosyx family hypercholesterolemia (HoFH), which affects approximately 11,000 patients worldwide. REGENXBIO previously announced completed dosing of the expanded cohot in phase 2 I/II trials, including RGX-501 steroid prevention. 13 weeks steroid treatment. Serious side effects or significant elevations in liver enzyme levels were reported in the extended cohot 2. REGENXBIO plans to assess low density intelligent protein (LDL-C) levels in Extended Cohot 2 after all patients have completed steroid therapy and expect to provide interim data in the first half of 2020. REGENEXBIO is also working to proceed with RGX-181, which has been fda-designated as a rare pediatric disease and orphan drug as a candidate for the treatment of the late infant neurocloidal lipopusrinosis second (CLN2) disease. The company expects to submit a investigative new drug (IND) application to the U.S. Food and Drug Administration (FDA) in the second half of 2020 to enable the start-up of the first human clinical trials for children with CLN2 disease. RGX-111 is a potential treatment for REGENEXBIO for another rare disease called mysmosis type 1 I (MPS I), a rare and high-retical genetic disease that can cause pox, spinal cord compression and cognitive impairment. Recruitment, screening and further on-site activation are underway in phase I clinical trials evaluating RGX-111 for the treatment of MPS I. RGX-111 has received orphan medicines, rare pediatric diseases, and fast-track designations from the FDA. Program updates will be released in the second half of 2020. REGENXBIO'S PIPELINE REPRESENTS A BALANCED APPROACH PURSUED BY MANY LIFE SCIENCES COMPANIES, FOCUSING ON RARE DISEASES AND POTENTIAL BLOCKBUSTER TREATMENTS (WET AMD). Cerecor, Inc. (CERC), Rockville, MD Cerecor, Inc. is a publicly traded biotechnology company focused on developing innovative treatments for orphan diseases and very rare pediatric diseases. Founded in 2011, the company has launched a number of prescription drugs, prescription medical devices and dietary supplements on the market. Cerecor's pipeline includes many rare disease treatments in the early stages of development. It is an ultra-water oral formulation of D-galactose currently being developed for the treatment of CERC-801, infoglucomutase 1 (PGM1) deficiency, also known as PGM1-CDG, a genetic disorder with a wide range of symptoms. This potential treatment is in Phase I clinical trials and is a pediatric program with prompt 505(b)(2) pathway assignment. Other pediatric rare diseases targeted by Cerecor include the rare, congenital disorder MPI-CDG in stage I pediatric trials with urgent 505 (b)(2) pathway assignment; White blood cell adhesion bonding type II (LADII), also known as SLC35C1-CDG, which is in the IND support phase; Ultra-rare mitochondrial DNA depletion syndrome (MDS) also known as Deoxyguanosine kinaze (DGUOK) deficiency in IND activation Asklepion Pharmaceuticals, Baltimore, MD Asklepion Pharmaceuticals is a biopharmaceuty company focused solely on developing rare disease treatments, especially for rare pediatric diseases without viable treatment. The company has shifted its focus only to pediatric rare diseases in recent years. Asklepion was founded in 2006 to commercialize a 15-year academic program focused on the development of bile acids for fetal errors in bile acid metabolism, a rare genetic disorder. The main treatment candidate is for acute lung damage induced by cardiopulary bypass surgery (CBP), which recently completed phase III clinical trial registration in July 2019. Cardiopulondy bypass surgery can damage lung tissue and blood vessels, which can lead to significant postoperation problems. The treatment of Asklepion is unique in that it is prevention rather than prevention. By intravenously administering pre-protein building block L-citrulline surgery, Asklepion believes there are postoperoperal complications caused by CBP. The treatment was fda orphan drug designation in 2017. We also started clinical trials for sickly cell disease (acute setting) in the second quarter of 2020. Lead-on Biosciences, Gaithersburg, MD For more than three years, the company's iteration, now known as Radiant Biosciences, has shown a firm commitment to treating and helping ADA-SCID patients and their families. Bubble boy's disease - or adenosine deaminase deficiency - severely combined immunodeficiciulity (ADA-SCID) - is very rare, a very rare, inherited autoimmune disorder that damages the immune system and can be fatal if untreated. In October 2018, Radiant Biosciences, formerly known as Sigma Tau Pharmaceuticals, received FDA approval for REVcovi™, which provides enhanced care to patients residing in ADA-SCID. About 1 in 200,000 to 1,000,000 newborns are affected worldwide, representing about 15% of all SCID cases (Source: NIH). As an extremely rare disease, the patient population is a minority: Radiant currently treats 35 ADA-SCID patients in the United States and seven ADA-SCID patients in Canada. Based in Gaithersburg, Maryland, Radiant's roots are deep in the treatment of rare and very rare diseases. Radiant Biosciences, Inc. is a subsidiary of Radiant S.p.A., dating back to Sigma-Tau, a family-owned pharmaceutical company founded in Italy in 1957 by Emilio and Dr. Claudio Cavazza. Sigma Tau Pharmaceuticals was a pioneer in the treatment of rare diseases and received its fourth orphan drug designation in 1984. Today, Radiant's sole focus is on researching, developing and deploying new treatments for patients suffering from some of the world's rarest diseases. Revcovi's recent FDA approval for ADA-SCID treatment provides 8 rare disease products that lead to North America. Canada) market. With a deep commitment to advancing a diverse pipeline of new drug and treatment candidates, Radiant has seven clinical phase programs in the pipeline. The two programs in the Phase III trial include Cerebrotendinous Xanthomatosis (CTX) for the treatment of GNE Myopathy and LB101 for the treatment of LB301. Several other rare disease-focused BHCR companies are ready to emerge. Baltimore's Non-Drug Therapy, MD, is a biotechnology company looking for a cure through stem cell biology. The pipeline currently has four rare disease treatments in the pipeline, all of which are in the early stages of development. The company recently announced a $4M Series A round. Another biotechnology based in Baltimore, Abri Sciences, recently launched in September 2019. Abri Science is focused on developing PKU therapeutics with licensed technology. Since its launch, Abri Science has received a $225,000 Small Business Technology Transfer (STR) award from the U.S. National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK). BHCR has rapidly become a global hub for the development of rare disease drugs. A host of BHCR companies including many cell and gene therapy companies have made rare disease drug development an important component of their pipeline strategy. BioBuzz will feature rare disease stories during Rare Disease Awareness Month. And as we move deeper into 2020, we will keep a close eye on companies focused on these rare diseases. Check back for the latest news and developments. The following two tabs make changes to the following: Below.