



## CSL Behring and Seattle Children's Research Institute to Advance Gene Therapy Treatments for Primary Immunodeficiency Diseases

SEATTLE and KING of PRUSSIA, Pa.

Initially, the alliance will develop treatment options for patients with two rare, life-threatening primary immunodeficiency diseases -- Wiskott-Aldrich Syndrome (WAS) and X-linked Agammaglobulinemia (XLA)

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**SEATTLE and KING of PRUSSIA, Pa. – June 02, 2020** - Seattle Children's Research Institute, one of the top pediatric research institutions in the world, and global biotechnology leader CSL Behring announced a strategic alliance to develop stem cell gene therapies for primary immunodeficiency diseases.

Initially, the alliance will focus on the development of treatment options for patients with two rare, life-threatening primary immunodeficiency diseases -- Wiskott-Aldrich Syndrome and X-linked Agammaglobulinemia. These are two of more than 400 identified primary immunodeficiency diseases in which a part of the body's immune system is missing or functions improperly.

"CSL Behring will collaborate with Seattle Children's experts to apply our novel gene therapy technology to their research pipeline, with an aim to address unmet needs for people living with certain rare primary immunodeficiency diseases," said Bill Mezzanotte, MD, Executive Vice President, Head of Research and Development for CSL Behring. "Expanding our gene therapy portfolio into an area of immunology well known to CSL exemplifies how we are strategically growing our capabilities in this strategic scientific platform and are collaborating with world class institutions to access innovation with the potential to vastly improve patients' lives."

"Stem cell gene therapies that correct the genetic abnormality driving a child's disease will transform the therapeutic options for children with Wiskott-Aldrich Syndrome, X-Linked Agammaglobulinemia and other immunodeficiency diseases," said David J. Rawlings, MD, director of the Center for Immunity and Immunotherapies and division chief of immunology at Seattle Children's, and a professor of pediatrics and immunology at the University of Washington School of Medicine. "The collaboration with CSL Behring supports our longstanding research programs for pediatric immunodeficiency diseases and will accelerate this research toward clinical trials, helping get these innovations to the children who need them."

CSL Behring researchers, working with researchers from Seattle Children's Research Institute, will investigate applying the proprietary platform technologies, Select+™ and Cytegrity™, to several pre-clinical gene therapy programs. These technologies, which have broad applications in ex vivo stem cell gene therapy, are designed to address some of the major challenges associated with the commercialization of stem cell therapy, including the ability to manufacture consistent, high-quality products, and to improve engraftment, efficacy and tolerability.

Wiskott-Aldrich Syndrome (WAS) has an estimated incidence between one and 10 cases per million males worldwide, according to the National Institutes of Health. WAS patients' immune systems function abnormally, making them susceptible to infections. They also experience eczema, autoimmunity and difficulty forming blood clots, leaving them vulnerable to life threatening bleeding complications. Today the only known cure for WAS is a stem cell transplant, if a suitable donor can be found.

X-linked Agammaglobulinemia (XLA) is another rare primary immunodeficiency in which patients have low levels of immunoglobulins (also known as antibodies),

which are key proteins made by the immune system to help fight infections. Like WAS, XLA affects males almost exclusively, although females can be genetic carriers of the condition. While no cure exists for XLA, the goal of treatment is to boost the immune system by replacing missing antibodies and preventing or aggressively treating infections that occur, according to the Immune Deficiency Foundation.

### ***About Seattle Children's***

Seattle Children's mission is to provide hope, care and cures to help every child live the healthiest and most fulfilling life possible. Together, Seattle Children's Hospital, Research Institute and Foundation deliver superior patient care, identify new discoveries and treatments through pediatric research, and raise funds to create better futures for patients.

Ranked as one of the top children's hospitals in the country by *U.S. News & World Report*, Seattle Children's serves as the pediatric and adolescent academic medical center for Washington, Alaska, Montana and Idaho – the largest region of any children's hospital in the country. As one of the nation's top five pediatric research centers, Seattle Children's Research Institute is internationally recognized for its work in neurosciences, immunology, cancer, infectious disease, injury prevention and much more. Seattle Children's Hospital and Research Foundation works with the Seattle Children's Guild Association, the largest all-volunteer fundraising network for any hospital in the country, to gather community support and raise funds for uncompensated care and research. [Join Seattle Children's](#) bold initiative – It Starts With Yes: The Campaign for Seattle Children's – to transform children's health for generations to come.

For more information, visit [seattlechildrens.org](https://seattlechildrens.org) or follow us on [Twitter](#), [Facebook](#), [Instagram](#) or on our [On the Pulse](#) blog.

### ***About CSL Behring***

CSL Behring is a global biotherapeutics leader driven by its promise to save lives. Focused on serving patients' needs by using the latest technologies, we develop and deliver innovative therapies that are used to treat coagulation disorders, primary immune deficiencies, hereditary angioedema, inherited respiratory disease, and neurological disorders. The company's products are also used in cardiac surgery, burn treatment and to prevent hemolytic disease of the newborn. CSL Behring operates one of the world's largest plasma collection networks, CSL Plasma. The parent company, [CSL Limited](#) (ASX:CSL;USOTC:CSLLY), headquartered

in Melbourne, Australia, employs more than 26,000 people, and delivers its life-saving therapies to people in more than 70 countries. For more information, visit [www.cslbehring.com](http://www.cslbehring.com) and for inspiring stories about the promise of biotechnology, visit Vita [www.cslbehring.com/Vita](http://www.cslbehring.com/Vita).

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