Breakthrough Rare-Disease Therapy Gives Children The Opportunity To Live Healthy Lives

Indiana University

A breakthrough therapy that can treat two rare childrens' diseases has been brought to market thanks to the efforts of Indiana University faculty and its Innovation and Commercialization Office (ICO)._

Burosumab, brand name Crysvita, has orphan drug designation for x-linked hypophosphatemia, a rare inherited form of rickets that affects children aged one year or older. It can also be used to treat another rare disease, tumor-induced osteomalacia, that causes bone malformation. Burosumab highlights how the drug development process works to successfully bring a new treatment to market after decades of research.

Michael Econs and Kenneth E. White, of the Indiana University School of Medicine in Indianapolis, led the research that resulted in the development of a novel treatment for the debilitating disease X-Linked Hypophosphatemia (XLH). XLH is a deforming bone disorder that causes rickets, or softening of the bones, and other complications.

The treatment is based on Econs' and White's initial discovery of Fibroblast Growth Factor-23 (FGF23). Patients with the

disease overproduce FGF23. The hormone blocks phosphate absorption in the kidneys and intestines, resulting in low levels of phosphate in the blood, which negatively affects bone development. Crysvita is a monoclonal antibody that binds FGF 23 and inhibits its excessive activity within the body, allowing phosphate absorption by the body and normal constitution of bones in children.

"I first started seeing XLH patients in 1986 and started doing research on XLH shortly thereafter," said Econs, who is Distinguished Professor of Medicine and Medical and Molecular Genetics at the IU School of Medicine. "When Burosumab was finally approved, it was like a dream come true."

ICO manages the FGF 23 IP portfolio and related agreements and negotiated a license with Kyowa Kirin Co., Ltd., a Tokyo-based pharmaceutical and biotechnology company to advance the technology to the clinic. Kyowa Kirin discovered and manufactured burosumab, based on this licensed technology, and entered into a collaboration and license agreement with Ultragenyx Pharmaceutical of Novato, Calif., to develop and commercialize the drug.

Crysvita obtained U.S. Food and Drug Administration (FDA) approval in 2018. In 2020, the FDA also approved Crysvita for the treatment of tumor-induced osteomalacia, highlighting another success for the FGF23 technology. The treatment is an injection delivered every two weeks based on the weight of the patient.

The technology was developed with support from National Institutes of Health (NIH) research grants and is a clear example of the positive outcomes that basic research can have on the well-being of patients and the public at large.

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