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# Patients with treatment-resistant autoimmune blood conditions may benefit from sirolimus

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The immunosuppressant sirolimus is an effective and safe steroid-sparing therapy for children and young adults with highly treatment-resistant autoimmune blood conditions, according to a study published online today in *Blood*, the Journal of the American Society of Hematology (ASH). This treatment is particularly effective in children with autoimmune lymphoproliferative syndrome (ALPS), a chronic genetic disorder characterized by the buildup of white blood cells in the organs.

Patients with ALPS and other autoimmune disorders often have immune systems that destroy their body's own healthy blood cells. The resulting decrease in blood cells causes symptoms such as anemia, uncontrolled bleeding, and infection. Few effective and well-tolerated therapies exist to manage these chronic autoimmune issues. While standard immunosuppressive therapy with corticosteroids may help some patients, others are resistant, intolerant, or cannot successfully maintain healthy blood cell counts when they discontinue medication. Recently, investigators reported that sirolimus successfully resolved these autoimmune conditions in a small group of children with ALPS without causing adverse side effects.

"Patients with ALPS and other chronic autoimmune disorders have few long-term treatment options for managing harmful conditions related to reduced counts of red blood cells, platelets, or white blood cells," said senior study author David Teachey, MD, of the Children's Hospital of Philadelphia. "Corticosteroids are not a permanent solution, as they are often associated with long-term health effects, such as osteoporosis and higher risk of infection."

To improve disease management for patients with ALPS and similar autoimmune disorders, a research team led by Teachey and his colleague Karen Bride, MD, PhD, conducted a multicenter clinical trial evaluating sirolimus in 30 patients ranging from 5 to 19 years old. All study participants were either intolerant or resistant to corticosteroids. Patients received 2 mg/m2 to 2.5 mg/m2 per day of sirolimus in either liquid or tablet form for six months. After six months, those who benefited from the drug were allowed to continue treatment with continued follow-up appointments to monitor toxicities.

Researchers demonstrate that 11 of the 12 children with ALPS achieved normal blood cell counts within one to three months of starting sirolimus. The remaining patient achieved a complete response by 18 months. Additionally, all ALPS patients successfully weaned off steroids and discontinued all other medications within one week to one month after starting sirolimus. Patients remained on treatment for a median of 3.5 years. In addition, investigators observed that patients suffered few adverse side effects after a median follow-up of two years.

While the majority of study participants with similar autoimmune disorders also achieved complete response (8 of 12 patients), it did not occur until three months after start of treatment. Based on these findings, the authors propose use of sirolimus as early therapy for patients with these chronic treatment-resistant autoimmune blood conditions, especially for those with ALPS.

"This study demonstrates that sirolimus is an effective and safe alternative to steroids, providing children with an improved quality of life as they continue treatment into adulthood," said Dr. Teachey. "While further studies are needed, sirolimus should be considered an early therapy option for patients with autoimmune blood disorders requiring ongoing therapy."

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Source:
American Society of Hematology

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