

September 2022

On Friday September 14th, the [FDA approved gene therapy for early active cerebral adrenoleukodystrophy](#). Dr. Florian Eichler and team partnered with Boston Children's Hospital and bluebird bio to see this come to fruition.

"It feels like a milestone so many at MGH have contributed to over the past decade. Patricia Musolino was a steadfast partner in recruiting patients from Latin America and has advanced new imaging insights. Nurse Practitioner Catherine Becker helped care for and monitor these patients. A dozen research coordinators worked tirelessly on this, including Razina Aziz-Bose who helped launch the trial and is currently a child neurology resident at MGH. Over the years many others contributed such as Camille Corre, Haley Andonian, Daniel Kelly and Melissa Bamberg who helped complete a final regulatory inspection. MGH neuropsychologists tested boys over the decade and hundreds of brain MRIs were performed with MGH anesthesia and neuroradiology. We are so proud to see this all come to fruition," said Dr. Eichler.

"For decades we have seen boys with ALD struck with devastating brain demyelination in the prime of their development," he continued. "This transformative therapy comes at a time when we have begun screening for ALD in newborns in many states including Massachusetts, and it provides much needed hope for families and care teams invested in identifying brain disease early, when it is most treatable."

We look forward to seeing this option positively impact families affected by CALD.

See article from June here:

[New Therapy for Cerebral ALD Recommended for Approval](#)

