Riaan Research Initiative
Transforming the Future of Cockayne Syndrome

August 2026

# From Hope to Reality

## Five-Year Impact Report

RIAAN RESEARCH INITIATIVE

# Letter From Our Founder & Board Chair

## Jo Kaur

Five years ago, when we launched Riaan Research Initiative (RRI), Richie and I had trouble envisioning the future. The task ahead felt almost impossible for two grieving parents whose firstborn child, Riaan, had just been diagnosed with a fatal genetic disease: Cockayne syndrome, CSA/ERCC8, Type II.

We knew nothing about building a research nonprofit or developing a gene therapy. We didn't know whether we could raise the millions it would take, or whether science could move quickly enough to matter for Riaan and other children.

**What we did know was that doing nothing was not an option.**

Less than three months after RRI's founding, on September 4, 2021, the organization received its 501(c)(3) tax-exempt status. What began as two parents searching desperately for a way forward had become an organization with a mission: to accelerate research and the development of treatments for Cockayne syndrome.

Five years later, it is difficult to comprehend how far that mission has taken us.

We are incredibly proud of our team and deeply grateful to our Board of Directors, our Scientific Advisory Board, our advisors, the many volunteers and supporters who have selflessly given their time, energy, and resources to help us fundraise and advance our mission, and Cockayne syndrome families from around the world who have worked alongside us to help ensure that our children are not left without treatments.

This Five-Year Impact Report captures snapshots of that work: the defining moments, milestones, and headlines that tell the story of how far we have come and the extraordinary community that made it possible. It is impossible to capture the full story in these pages, but in due time, we hope to share it all. Thank you for being part of this complex, wild, and miraculous journey with us. Together, we will cure Cockayne syndrome.

Over

# $4M

Raised

## A Tiny Community A Mighty Push

**FIRST GENE THERAPY**
RRI helped drive, develop, and fully fund the first CSA/ERCC8 gene therapy program from concept to clinic, spanning preclinical development, manufacturing, toxicology, and regulatory review.

**OUR STORIES, TOLD OUR WAY**
We made storytelling a cornerstone of our advocacy, sharing the realities of Cockayne syndrome, rare disease, drug development, grief, hope, and our families' lives through Riaan's Newsletter and beyond.

**DRUG REPURPOSING PROGRAMS**
RRI launched two groundbreaking research programs to identify existing small molecules with therapeutic potential for Cockayne syndrome, CSA/ERCC8 and CSB/ERCC6.

**GLOBAL PATIENT REGISTRY**
RRI, in partnership with Sanford CoRDS, built a patient registry to collect critical clinical data, deepen understanding of the disease, and support future research and clinical trials.

**MEDIA & AWARENESS**
RRI brought the patient community and the power of parent-led drug development to audiences around the world through local, national, and international media coverage, interviews, webinars, podcasts, and long-form storytelling.

**A WORLD-CLASS NETWORK**
RRI built a global network of leading scientists, clinicians, drug developers, and rare disease experts committed to advancing treatments for Cockayne syndrome.

**FIRST PATIENT TREATED**
On April 21, 2026, 6-year-old Riaan Singh Digeorge became the first child in the world treated with gene therapy for Cockayne syndrome.

RIAAN RESEARCH INITIATIVE

# From impossible to FIRST Patient

## CS Gene Therapy From Concept to Clinic

**2021: RIAAN RESEARCH INITIATIVE IS BORN**
RRI launches with an urgent mission: to accelerate treatments for Cockayne syndrome.

**2021 to 2022: BUILDING THE SCIENCE**
RRI brings together researchers, clinicians, CS disease experts, and partners to begin building a gene therapy program for Cockayne syndrome.

**2022 to 2023: PROOF OF CONCEPT**
Preclinical studies in mouse models advance the science and provide critical evidence supporting development of a gene therapy for Cockayne syndrome.

**2023: PRE-IND MEETING WITH FDA**
RRI's gene therapy program reaches an important regulatory milestone with a Pre-IND meeting with the U.S. Food and Drug Administration, helping define the path toward human treatment.

**2024: TOWARD THE CLINIC**
The program moves into formal development, including the studies and planning required to support human treatment.

**2025 to 2026: TOXICOLOGY STUDY**
A pivotal GLP toxicology study is completed, generating critical safety data needed to support regulatory review.

**2025: CLINICAL MANUFACTURING**
Clinical-grade, self-complementary AAV9 (scAAV9) gene therapy is manufactured and tested under GMP standards for patient use.

**JANUARY 25, 2026: IND FILED WITH THE FDA**
Years of drug development culminate in the preparation and submission of a comprehensive Investigational New Drug application to the FDA.

**FEBRUARY 24, 2026: IND CLEARED**
Following a rigorous 30-day review and Information Requests, the FDA clears the IND, allowing first-in-human dosing to proceed.

**APRIL 21, 2026: FIRST PATIENT TREATED**
Riaan becomes the first child in the world treated with gene therapy for Cockayne syndrome via an intracerebroventricular (ICV) neurosurgical procedure.

RIAAN RESEARCH INITIATIVE

# The first patient is just the BEGINNING

Pending regulatory authorization and funding to cover clinical costs, our goal is to help ensure more children with Cockayne syndrome have the opportunity to receive treatment with *scAAV9-CSA.*

**BUILDING WHAT COMES NEXT**

At the same time, we continue to invest in the future of Cockayne syndrome research and therapeutic development. We are committed to building on the groundbreaking work of the first gene therapy program, advancing next-generation approaches, exploring additional therapeutic strategies, and creating more possibilities for children and families affected by this devastating disease.

**THE THERAPY IS READY**

The clinical gene therapy product is already manufactured with enough doses for additional children.

**OUR IMMEDIATE GOAL**

Help bring *scAAV9-CSA* to more children pending regulatory authorization and the funding needed to cover clinical costs.

**OUR NEXT CHAPTER**

Create more therapeutic possibilities for children with Cockayne syndrome around the world.

***In five years, your support helped us turn an ambitious vision into a treatment.***

**Fierce commitment. Relentless execution.**
**Support our next chapter and help bring treatments to more children with Cockayne syndrome.**

**DONATE TODAY**

RIAAN RESEARCH INITIATIVE

riaanresearch.org/support