Who WE ARE

BDSRA foundation
Batten Disease Support + Research + Advocacy

OUR MISSION

BDSRA is determined to provide unwavering support, fund progressive research, and be a source of steadfast advocacy for all CLN types. Founded in 1987, Batten Disease Support, Research, & Advocacy Foundation (BDSRA) is the largest international nonprofit organization in North America. Our long-term vision is a world without Batten disease.

BATTEN DISEASE is a rare, fatal inherited disorder of the nervous system and has no known cure. Those affected with Batten disease often suffer progressive neurological impairment, seizures, blindness, and loss of speech and motor skills. Batten Disease, or Neuronal Ceroid Lipofuscinosis (NCL), is a family of lysosomal storage disorders which cause the build-up of fats and proteins (lipofuscins) in the body, causing cell death. There are 13 identified forms of the disease (CLNs), which are most commonly diagnosed between infancy and school age. Kufs, Parry and ANCL disease are known adult forms of Batten.

Hundreds of families cope with the diagnosis of Batten Disease every year through education, hope, and a shared determination to find a cure.

Did you know? International Batten Disease Day is June 9 annually.

To learn more about Batten disease, visit:

Though recent improvements in genetic testing have made diagnosing Batten disease much quicker and more reliable, many families experience long diagnostic journeys. Autism, seizure disorder, epilepsy, Pervasive Developmental Disorders, and others are common among roughly 30 early misdiagnoses. According to the Centers for Disease Control, 2–4 births per 100,000 in the U.S. are affected by Batten disease, though some researchers in the field suggest these numbers are low.

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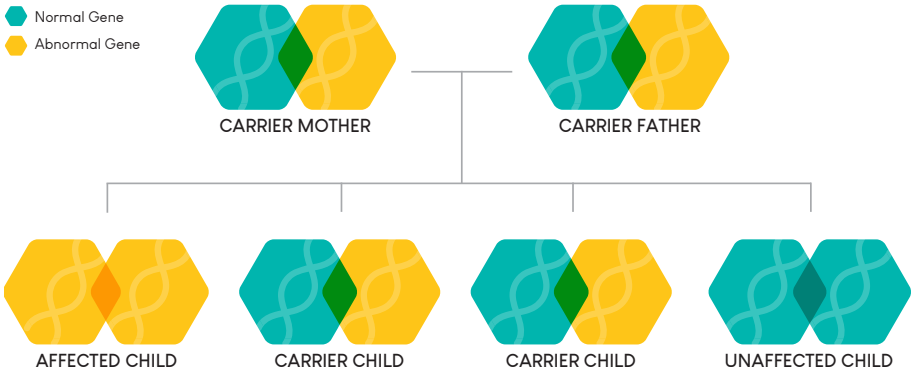
The Genetics of BATTEN DISEASE

Batten disease, for the most part, is an autosomal recessive disease. Autosomal recessive diseases require two copies of an abnormal gene to be passed down in order for the disease to develop. This means that both parents must be carriers in order to have the possibility of their child inheriting a recessive condition. The figure below illustrates the different inheritance possibilities.



When two carriers have children, **at each pregnancy** there is a one in four chance that the child will be born with autosomal recessive Batten disease.

If the **child receives both abnormal** genes

25%

chance this **child is affected** with the disease

If the **child gets a normal gene** from one parent and **an abnormal gene** from the other parent

50%

chance this **child is not affected** but **is a carrier** of the disease

If the **child receives both normal** genes

25%

Chance this **child is not affected** and is not a carrier

What Can You DO TO HELP?

- Recognize June 9 as International Batten Disease Awareness Day in your state.
- Support Recommended Uniform Screening Panel (RUSP) alignment in your state.
- Support cell and gene therapy.
- Support appropriations for 2024 Rare Disease Programs.
- Sign the letter for the Food and Drug Administration Task Force on Rare Disease Activities.
- Co-sponsor the BENEFIT Act.
- Join the Rare Disease Congressional Caucus.
- Support accelerated approval pathways.
- Subscribe to our monthly newsletter, *The Illuminator*.

Subscribe to our newsletter here:

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