

About the URBC

The University of Rochester Batten Center (URBC) is dedicated to learning about Batten disease and working towards finding treatments that can benefit affected children and families.

Formed in 2002, the URBC seeks to advance knowledge about the cause, process and clinical impact of Batten disease in order to develop and test promising therapeutic interventions in clinical trials.

The URBC developed the Unified Batten Disease Rating Scale (UBDRS) to measure characteristics of Batten disease, such as movement, behavior, seizures, ability to complete daily activities, and general well-being.

JUMP is the URBC's first clinical trial in Batten Disease

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The **URBC** partners with the National Institutes of Health (NIH), the Food and Drug Administration (FDA), the Batten Disease Support and Research Association (BDSRA), and others to carry out its research activities. Since its inception, the URBC has actively published its research efforts in internationally recognized peer-reviewed journals to make their progress in Batten disease available to the public.

For more information about the URBC, including a list of publications, please visit the URBC's website at:

<http://www.urmc.rochester.edu/neurology/batten-disease-center/>

For more information about the JUMP study, please contact:

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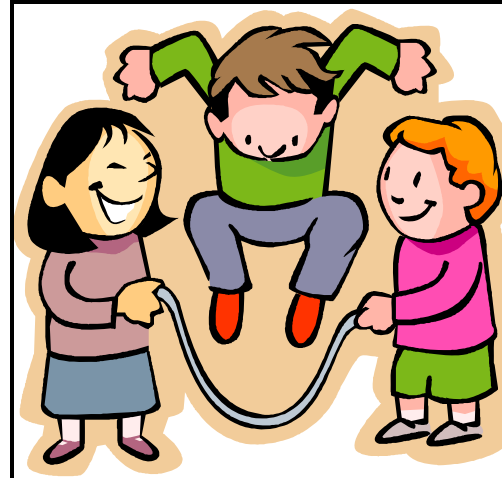


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JUVENILE MYCOPHENOLATE PHASE II CLINICAL TRIAL IN BATTEN DISEASE



University of Rochester Batten Center (URBC)

RSRB-University of Rochester-Approval
RSRB No. 33940
Expires November 15, 2011
- cj 7/13/11-



What is the JUMP study?

The JUMP study is a clinical trial to learn if mycophenolate (CellCept) is safe and well tolerated in children with Juvenile Neuronal Ceroid Lipofuscinosis (JNCL), also known as Batten disease.

Another purpose of the study is to see if mycophenolate (CellCept) has an effect on the symptoms of JNCL. These symptoms include unusual movements, seizures, problems with learning or behavior, or difficulty performing daily tasks.

What is Mycophenolate Mofetil (CellCept)?

Mycophenolate mofetil (CellCept) is a medication that suppresses the immune system, meaning that it makes the body less able to fight off infection or illness. It is approved by the US Food and Drug Administration (FDA) to prevent rejection of transplanted organs in both children and adults, but is not approved for use in treating JNCL. Therefore, it is considered an investigational drug for JNCL.

The JUMP study will be the first clinical trial to date for Batten disease. There will be 30 children in the study at up to 30 clinical sites in the United States. Each child will be in the study for 22 weeks.

What is a clinical trial?

- A clinical trial is a research study in human volunteers to answer specific health questions.
- Carefully conducted clinical trials are the fastest and safest way to find treatments that work and ways to improve health.
- Clinical trials determine if experimental treatments or new ways of using known therapies are safe, tolerable and effective under controlled environments.

In order to participate:

- Must have JNCL that is confirmed by genetic testing and clinical examination
- Must be able to walk 10 feet without help beyond that required due to vision loss
- Must have a local doctor who is willing to conduct the study
- Must have a parent/legal guardian willing to accompany the child to study visits, both locally and to the URBC in Rochester, NY
- Must be able to swallow liquid medication
- Cannot have ever taken mycophenolate mofetil (CellCept).

During the study:

- The study is 22 weeks long.
- During this time, there will be 12 study visits, including 4 visits to the URBC in Rochester, NY, 4 visits to your local doctor's office and 4 visits by telephone.
- Visits will include interviews, physical and neurologic exams, blood and urine collection, and tests of thinking, memory, learning, movement, and behavior.
- Travel expenses are paid by the study.

Are there risks involved?

The most common side effects of mycophenolate mofetil (CellCept) in children include:

- Stomach upset (such as diarrhea, stomach discomfort, or nausea). Your child will be given Zantac throughout the study to minimize this risk.
- Decrease in blood cell counts
- Increased chance of infection
- Sore throat, fever, and/or colds
- Increase in blood pressure

If your child experiences side effects, the study doctor may recommend lowering the dose of study medication or stopping all study medication. Even if medication is stopped, your child would still be in the study. There are some risks when blood is drawn. These risks are further explained in the parent permission form.

The research doctor and team can also answer any questions you might have.

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