

CYSTINOSIS PATIENT RESOURCES

This resource list has been compiled by the Cystinosis Research Foundation for cystinosis patients and their families as a quick reference to assist you with your questions concerning insurance, and the process necessary to obtain medications and prescriptions. This information is intended for general education and should not be construed as advising on diagnosis or treatment of this or any other medical condition.

PROCYSBI™

www.procysbi.com/

PROCYSBI™, approved by the FDA on April 30, 2013, is the first new cystine-depleting medication for nephropathic cystinosis in nearly 20 years.

PROCYSBI™ works by continuously reducing the toxic accumulation of cystine, and it can provide continuous control of cystine levels when taken every 12 hours and plays an important role in the management of nephropathic cystinosis.

Raptor Pharmaceuticals is the distributor of the PROCYSBI™ medication.

Patients must make an appointment with their doctor to determine if PROCYSBI™ is right for them. **Patients are strongly encouraged to enroll in RaptorCares™, a patient support program, created by Raptor Pharmaceuticals in preparation for their doctor's visit regarding PROCYSBI™ prescriptions.**

Enroll at www.RaptorCares.com/QA3 or call 855-888-4004.

RAPTORCARES™

www.RaptorCares.com

RaptorCares™ is a patient-support program, created by Raptor Pharmaceuticals, with input from nephropathic cystinosis patients and caregivers who understand the challenges and successes you may experience.

RaptorCares™ offers important information about nephropathic cystinosis as well as valuable tools to help you better manage the challenges along the way.

As of January 1, 2014 PROCYSBI™ is covered in all 50 states with Medicaid.

RaptorCares can assist patients to navigate the Medicaid approval process. RaptorCares does not (or cannot) provide co-pay assistance, but the co-pays for Medicaid patients are typically \$4-\$5 per prescription and no more than \$10.

CYSTAGON®

Cystagon® capsules contain cysteamine bitartrate, a cystine depleting agent that lowers the cystine content of cells in patients with cystinosis.

CVS ProCare is the sole distributor for Cystagon®

Contact CVS ProCare:

ProCare.AD@cvsprocare.com
888-700-0024

Hours of Operation (EST):
Monday – Friday: 8 a.m. – 8 p.m.
Saturday: 8 a.m. – 1 p.m.

CVS ProCare provides the following services:

Counseling on ordering Cystagon®
Ordering other medications
Insurance inquiries
Trained pharmacist assistance available 24 hours

CYSTARAN™ (EYE DROPS)

www.cystaran.com

CYSTARAN™ is the only FDA-approved ophthalmic therapy for corneal crystals in cystinosis patients.

For information about CYSTARAN™ call Accredo Specialty Pharmacy at: 800-440-0473.

To contact Sigma-Tau, call Lesli King, Senior Manager, Patient Affairs: 301-670-5450 or lesli.King@sigmatau.com.

Prescribing information is available at www.cystaran.com

NIH: NATIONAL INSTITUTES OF HEALTH

www.nih.gov

Appointments with Dr. William Gahl

Contact:

Joy C. Bryant, RN, BSN, CCRC
Research Nurse Specialist
301-443-8690, 102-10460 (page)
National Human Genome Research Institute
National Institutes of Health
9000 Rockville Pike
Building 10/Room 3-2551
Bethesda, Maryland 20892

CYSTINOSIS RESEARCH FOUNDATION (CRF)

www.cystinosisresearch.org

The Cystinosis Research Foundation is dedicated to finding better treatments to improve the quality of life for those with cystinosis and to ultimately find a cure for this devastating disease.

The Cystinosis Research Foundation issues grants for bench and clinical research studies bi-annually in order to accelerate research progress and ensure that cystinosis research is on-going and focused on novel treatments and a cure.

The Cystinosis Research Foundation is also dedicated to educating the public and the medical community about cystinosis to ensure early diagnosis and proper treatment.

Cystinosis Research Foundation
18802 Bardeen Avenue, Irvine, CA 92612
949-223-7610

CCIR: CURE CYSTINOSIS INTERNATIONAL REGISTRY

www.cystinosisregistry.org

CCIR is a collaborative effort by the leaders in the cystinosis community to identify people with cystinosis worldwide, to collect their medical history and information, and to share their de-identified (anonymous) information with the research community in an effort to accelerate novel treatments and a cure for cystinosis.

For questions contact:

Betty Cabrera, CCIR Curator

858-822-3747

email: curator@cystinosisregistry.org

CCIR PARTNERS AND ADVOCATES

Cystinosis Research Foundation

www.cystinosisresearch.org

Cystinosis Foundation

www.cystinosisfoundation.org

Cystinosis UK

www.cystinosis.org.uk

Cystinosis Australia

www.cystinosis.com.au/about.html

Cystinosis Foundation Germany

www.cystinose-selbsthilfe.de

Cystinosis Foundation Ireland

www.cystinosis.ie

Cystinosis Mexico AC

Cystinosis Support Group South Africa

www.cystinosis.co.za

Cystinosis Awareness & Research Effort

www.cystinosis.ca

Tina's Hope for a Cure

www.tinashopeforacure.org

Joshua's Journey of Hope

www.joshuasjourney.org

Jenna and Patrick's Foundation of Hope

www.jennaandpatrick.org

Hope for Holt

www.hopeforholt.org

24 Hours for Hank

www.24hoursforhank.org

NORD: NATIONAL ORGANIZATION FOR RARE DISORDERS

www.rarediseases.org

NORD's vision and guiding principles on which our advocacy initiatives are based:

- A national awareness and recognition of the challenges faced by people living with rare diseases and the associated costs to society.
- A nation where people with rare diseases can secure access to diagnostics and therapies that extend and improve their lives.
- A social, political and financial culture of innovation that supports both the basic and translational research necessary to create diagnostic tests and therapies for all rare disorders.
- A regulatory environment that encourages development and timely approval of safe and effective diagnostics and treatments for patients with rare diseases.

MEDICAL RESOURCES

Gene Tests

www.geneclinics.org

Clinical Genetic Testing

- www.cincinnatichildrens.org/service/s/star/genetic-testing/
Test must be ordered by a physician
- University of California, Irvine – Mitomed Diagnostic Laboratory
www.mammag.uci.edu/mitomed
- University of California, San Diego
www.cystinosiscentral.org

Kidney Transplants and Donation

www.kidney.org

OTHER RESOURCES

CheckOrphan

www.checkorphan.org

Genetic Alliance

www.geneticalliance.org

- U.S. Department of Health & Human Services
National Health Information Center
www.Healthfinder.gov

RARE: THE GLOBAL GENES PROJECT

www.globalgenes.org

The Global Genes Project is one of the leading rare and genetic disease patient advocacy organizations in the world. The non-profit organization is led by Team RARE and promotes the needs of the rare and genetic disease community under a unifying symbol of hope – the Blue Denim Genes Ribbon™.

Rare and genetic diseases affect 1 in 10 Americans, 30 million people in the United States, and 300 million people globally. Over 7,000 distinct rare diseases exist and approximately 80 percent are caused by faulty genes. The National Institutes of Health estimates that 50 percent of people affected by rare diseases are children, making rare diseases one of the most deadly and debilitating for children worldwide.

It is estimated that 95 percent of all rare diseases do not have a single FDA-approved drug treatment, and there are currently fewer than 400 treatments approved by the FDA for rare diseases.

According to NIH estimates it will take 10,000 years at the current rate of FDA approvals to find therapies for all people suffering from rare and genetic diseases.

