

What Is Mast Cell Connect?

Mast Cell Connect is an electronic patient registry created to advance the understanding of mastocytosis as a disease. It is a voluntary online registry to collect information from patients with mastocytosis on their experiences with the disease. The ultimate goal of Mast Cell Connect is to help speed the development of new treatments for people with mastocytosis.

The registry allows mastocytosis patients and caregivers to enter information about their experience living with the disease directly into a secure, web-based data collection tool. Those who participate in Mast Cell Connect will be able to view the de-identified summary of responses from other patients, and can choose to receive information about ongoing clinical trials and other related research studies.

You are invited to learn more and to consider participating in this important effort. By providing researchers with a database of detailed medical information about people with a specific disease, registries have proven to be a valuable tool in better understanding rare diseases like mastocytosis.

Questions?

For questions about the goals of the Mast Cell Connect registry, contact the study doctor at mastcellregistry@blueprintmedicines.com or at 617-714-6678.

For all other questions, contact the Mast Cell Connect registry coordinator at coordinator@mastcellconnect.org.

About Mastocytosis

Mastocytosis is a rare disease in which immune cells known as mast cells abnormally build up in the skin, bone marrow and other parts of the body. In healthy people, mast cells produce so-called mediators, like histamine, that help activate the immune system and appropriately direct disease-fighting blood cells. In mastocytosis, however, the abnormal build-up of mast cells leads to high levels of these mediators, and can cause symptoms that resemble allergies, including hives, flushing, shortness of breath and anaphylactic shock. The signs, symptoms and severity of mastocytosis vary widely, but in more severe cases, mast-cell accumulation in the organs results in organ function impairment.

In patients with mastocytosis, mast cells can accumulate in the skin (known as cutaneous mastocytosis, or CM) and/or in tissues including bone, bone marrow, liver, spleen and the gastrointestinal tract (known as systemic mastocytosis, or SM).

In almost all patients with SM, a genetic mutation known as KIT D816V is believed to be the root cause of the disease. The genetic mutation is not hereditary, and it is highly unusual for SM to run in families. Today, most treatments for SM are used to provide symptom relief due to limited treatment options, and there is no cure for the disease.



Mast Cell Connect Patient Registry

Together, we can advance
mastocytosis research





Why Join?

The more we understand about mastocytosis and the more people participate in research and clinical trials, the more we can help advance research and speed development of new treatments for mastocytosis.

Learn from other patients' experiences.

By participating, you will gain access to data and insights gleaned from other patients' responses that may be useful in better understanding your own disease.

Find out about clinical trials and other research studies.

You can sign up to be notified about clinical trials and other research studies that you may be eligible for based on the information you enter into the registry.

Advance research and speed development of new treatments. By improving our understanding of mastocytosis and its impact on patients over time, you can help spur the development of new potential treatments.

Getting Involved

Who can join?

People with a diagnosis of mastocytosis, including systemic mastocytosis (SM), cutaneous mastocytosis (CM) and their variants, are invited to join Mast Cell Connect. To join, you must be able to provide informed consent. Anyone under 18, or adults who cannot make their own medical decisions or would prefer to have someone else enter their information, must have a family member, medical caregiver, legal guardian or other designee to register on their behalf.

What does participating in the registry involve?

If you join Mast Cell Connect, you will be asked to complete a questionnaire about your experience living with mastocytosis, as well as to share medical records that describe your diagnosis, treatments, symptoms and changes in the disease over time. You may occasionally be asked additional survey questions, and to ensure the registry's accuracy, you will be asked to update your information a few times a year.

Who has access to Mast Cell Connect?

The broader medical community, including researchers, physicians, patient advocacy groups and companies engaged in mastocytosis research, can request access to the registry. All information in the registry is de-identified, meaning it has been stripped of information that could be used to identify you. As a participant, you have immediate access to the pool of de-identified survey answers.

Additional Resources

Here are more resources that you may find useful if you have mastocytosis, care for someone with mastocytosis, or would like to learn more about participating in clinical trials:



www.systemicmastocytosis.com

The Mastocytosis Society

www.tmsforacure.org

National Organization for Rare Diseases (NORD): Mastocytosis

www.rarediseases.org/rare-diseases/mastocytosis

European Competence Network on Mastocytosis

www.mastocytosis.eu

About the Sponsor

About Blueprint Medicines

Blueprint Medicines is a biotechnology company developing a new investigational treatment for systemic mastocytosis (SM). At Blueprint Medicines, we are motivated by one goal: to dramatically improve the lives of people with debilitating diseases. Our investigational therapies are currently in clinical studies for SM, gastrointestinal stromal tumors and hepatocellular carcinoma. For more information, please visit www.blueprintmedicines.com.

About PatientCrossroads

PatientCrossroads is a leader in building web-based patient registries designed to advance research and connect patients with researchers, advocates and industry organizations working to understand or treat specific diseases and conditions. For more information, visit www.patientcrossroads.com.

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