

The VWM Registry

The Amsterdam Leukodystrophy Center has developed an online global registry for patients with Vanishing White Matter (VWM). The purpose of the registry is to gain as much insight as possible into the course of the disease, both in terms of clinical features, disease burden and MRI. This database plays an important role in developing treatment strategies and making decisions about therapeutic trials. When research studies or clinical trials are initiated, the registry provides rapid information and access to eligible candidates. Therefore, it is helpful that as many patients as possible are part of the registry.

Who can participate in the registry?

Patients of all ages with genetically proven Vanishing White Matter, from all over the world.

What does participation in the registry mean?

When a patient signs up for the Registry, we first ask to sign informed consent. Informed consent gives us permission to collect information about you/your child. We then collect information about the clinical situation, MRI (s), and genetic test results by asking you for records and information. In addition, we send an email to provide access to an online registry, where questionnaires and score forms are available for you to complete.

Over time, we may send additional emails to invite you to share additional information via questionnaires. The frequency of the invitations to fill in the regular questionnaires and score forms depends on the age and are: 4 times a year (age 0-4 years), 2 times a year (4-8 years), or once a year (8 years and older). We send a maximum of 3 questionnaires and 3 score forms per invitation, which take a total of 30 minutes.

Participation in additional studies can be executed through the registry, such as a study on the burden of the disease on patients, parents, siblings and partners.

Once registered, you are free to participate or not in any study executed through the registry. No additional hospital visits are required. When an MRI is obtained for clinical purposes, we appreciate a copy.

How to register?

You can register for the online registration by sending an e-mail to registerVWM@amsterdamumc.nl. Upon receipt of your message, we will send you more detailed information about the Registry so that you can decide to participate or not. We will provide consent forms to be signed and returned to us.