

GSD1b Data Collection Program Powered by RARE-X

Printable Cheat Sheet

Before you Start:

Direct Link to GSD1b Portal on RARE-X = <https://gsd1b.rare-x.org/>

Web Browser - Please only use Google Chrome or Apple Safari.

Questions / Concerns / Feedback – Jamas LaFreniere at jamas@sophieshopefoundation.org

Tips and Tricks (Spots that seemed a little tricky):

Add Patient - After you have enrolled and created an account you will be brought to Rare-X homepage. It will appear blank but there is a tab that says, “Add Patient”. Click on that tab to get started. If you have more than one child with GSD1b you will do this for each child.

Expand Surveys - Once the patient is added and you are back on the home screen, next to the patient’s name all the way to the right, there will be a + sign that you will need to click on. This will populate all the available surveys to be filled out.

Important Notes and Instructions (Keep handy while in the platform):

Consenting – Please note, if you are a caregiver you will be asked to consent for yourself and then also for your child once you add them as the patient. If you have multiple children w/ GSD1b, you will need to consent for each of them. If you are a patient reporting for yourself, you will have just one consent.

Diagnosis Survey – In this section you are asked to input any diagnosis (aka symptoms). You are asked to enter them one at a time. For GSD1b we should all have neutropenia and hypoglycemia, therefore should enter those at a minimum. This is also where you could add IBD or Crohn’s if you also have that diagnosis / symptoms. Any other symptom or diagnosis you have could be included in this section, even if you don’t think or know its not related to GSD1b. You never know what we could learn when we put all the data together.

Health and Development Survey – This is a very important survey. Everything you answer yes to will then create a level 2 survey for that identified issue. The level 2 surveys can then be found on your home page after completing this survey. In this survey most GSD1b patients / caregivers will be selecting **Digestive, Growth, Immune, and Endocrine**. It’s okay to say yes to other issues / challenges, as well, we just want to make sure we check off Endocrine, because that is where a future “metabolic survey” will come from. By selecting these you will then have more surveys available to you after the General Health.

Endocrine Survey – This is a level 2 survey based on answering yes to Endocrine issues. It’s possible you will answer no to all the questions and that’s okay. However, at the bottom you can check “yes” for additional issues. **Please click Yes and then write in hypoglycemia**. This is important as it will help us capture a future survey focused on blood sugar / metabolism.

Frequently Asked Questions:

How long will this take? Your first time through should be 45-60 mins. This includes account creation, enrollment, consenting, then getting into all the surveys.

Can we do it on our mobile phones or tablet? Not at the moment. We suggest using a PC. There are future enhancements to the platform that will improve the functionality on mobile devices.

Do I have to live in the United States to participate? NO. This is global and you can fill this out anywhere in the world, but if you are outside the US you will have to agree to an additional consent question. The goal of this is to be a global database to represent the full GSD1b population.

What languages are available? Currently English. If you understand English (even if not your primary language) or have someone that can go through this with you and translate we encourage you to participate.

When will other languages be added? We don't have firm dates yet, but we do expect Spanish to be next and are expecting it to be added by the end of 2022. As we get more details, we will communicate with everyone. We are expecting most major languages to eventually be available.

Why aren't there specific questions about GSD1b? Please remember this is built for all rare diseases and uses very standard surveys. This was part of the reason we selected Rare X, because the data in is in a format that is research ready and can be understood across diseases. We are working with RARE-X to add a metabolism / blood sugar survey at some point. We will announce when that is available and hope people will go back in and fill it out.

How often are we expected to enter data? The goal would be for everyone to go in once a year and review and make any updates, especially if a new symptom has presented itself. If specific surveys that are relevant to GSD1b are added, we will communicate that with you and hope you would go in and fill out that specific survey. This will include when we do have a specific metabolism / blood sugar survey. Once you have enrolled the first time, every subsequent time will be significantly easier and less time since you no longer need to enroll or consent.

Who own's the data? You (the patient or caregiver) always have full control of your data.