# Type 1 Plasminogen Deficiency: What You Need to Know

## Type 1 Plasminogen Deficiency: The Diagnosis

Plasminogen deficiency type 1, or PLGD, is a rare genetic condition. This means your body doesn't make enough working plasminogen, a protein that's important for breaking down fibrin that is formed and promotes normal healing.

Because this protein is not at the normal expected level for your age, your body can develop thick, woody lesions on mucous membranes that irritate and inflame normal tissue. These most commonly affect the eyes and gums, but they can also occur in the nose, airways, ears, gastrointestinal tract, urinary tract, female reproductive tract, skin, and other organs.

One important thing to know is that this is a condition that affects many organ systems in your body. Even if your symptoms are present in one area today, you will need to be monitored on a regular basis to detect if there are any symptoms that may show other organ systems are also involved.

The good news is that there is now an FDA-approved treatment that replaces the missing plasminogen. Before this therapy became available, symptoms were treated with less effective treatments. Today, we can treat the underlying deficiency itself.

Key messages to emphasize:

- PLGD is genetic and lifelong.
- Symptoms vary widely from person to person.
- It is manageable with appropriate treatment.
- Early treatment helps prevent progressive tissue damage and repeated surgeries.
- Surgical removal of lesions alone often results in rapid regrowth unless the underlying deficiency is treated.

## Preparing for the First Infusion

Your first infusion is an important milestone. Most patients tolerate treatment very well, and we'll walk you through every step. The first dose is usually given in a monitored setting to make sure you do well with the treatment. This is most important in young children with symptoms in their respiratory tract. After that, many patients transition to home infusions.

Instructions on mixing and giving the treatment safely will be provided. Over time, many patients or caregivers become very comfortable administering treatment at home.

Patients should know:

- Infusions are given into a vein (intravenously).
- Treatment is typically administered every 2–4 days; treatment may be spaced out over time based on your symptoms and needs.
- Home infusion is possible after appropriate training.
- A caregiver or home infusion nurse may assist.
- Keeping an infusion log is encouraged.
- Periodic plasminogen levels may be monitored.

## Linking to the Ambassador Program: Find Your Community

You're not alone in this diagnosis. Because plasminogen deficiency is so rare, many patients find it very helpful to connect with someone who has gone through the same experience. The Patient Ambassador Program connects newly diagnosed patients with trained individuals or families living with plasminogen deficiency who can share practical advice and emotional support. This isn't medical care—it's peer support from someone who truly understands what living with this condition is like.

In addition to requesting a Patient Ambassador, you can also explore these Plasminogen Deficiency Foundation resources:

- Newly Diagnosed Toolkit
- Frequently Asked Questions Toolkit
- Monthly community Zoom webinars

# Guide for Health Care Teams: Ryplazim® Information

## How to Order Ryplazim®

1. Confirm the diagnosis of plasminogen deficiency type 1 with plasminogen activity and antigen levels.
2. Complete the Patient Enrollment/New Start Form available through the RYPLAZIM® Healthcare Professional website.
3. Submit the completed enrollment form to Kedrion.
4. Kedrion's patient support services assist with:
    - insurance authorization
    - specialty pharmacy coordination
    - nursing support
    - home infusion training
    - financial assistance programs when eligible.

Whenever possible, refer people with plasminogen deficiency to a Hemophilia Treatment Center (HTC), where multidisciplinary teams have expertise in factor replacement therapies, infusion education, insurance navigation, and long-term management of rare coagulation disorders.

## Side Effect Profile

Overall, RYPLAZIM® has been shown to be well tolerated.
Most side effects reported during clinical trials were mild to moderate.

The most common side effects include:

- abdominal pain or bloating
- nausea
- fatigue
- headache
- dizziness
- constipation
- dry mouth
- joint or back pain
- pain in the arms or legs
- bleeding from existing lesions

Rare but important risks include:

- allergic reactions, including anaphylaxis
- bleeding complications
- respiratory distress during healing of airway lesions due to tissue sloughing
- the theoretical risk of transmitting infectious agents because the medication is made from carefully screened human plasma.

## How to Describe Ryplazim® to Your Patient

RYPLAZIM® is the first FDA-approved treatment specifically designed for plasminogen deficiency type 1. Rather than simply treating the symptoms, it replaces the plasminogen protein. By restoring plasminogen levels, the medication helps the body clear abnormal fibrin deposits. This allows existing lesions to heal and helps prevent new ones from developing. Because your body naturally uses up plasminogen over time, treatment needs to be repeated regularly—typically every two to four days—to keep plasminogen levels in a range that helps resolve lesions that are present and prevent new lesions from forming. Most patients experience meaningful improvement in their disease with ongoing treatment, and many are able to transition to infusions at home after receiving proper training.

Think of plasminogen as one of your body's cleanup proteins. Without enough plasminogen, you build up areas of fibrin that cannot be broken down or removed. RYPLAZIM® replaces your body with normal amounts of functioning plasminogen to allow your body to function as it is designed to do.