



**International Gaucher Alliance**  
8 Silver Street | Dursley | Gloucestershire | GL11 4ND | UK

**Vesna Aleksovsk**  
Chair & Director  
Tel & Fax: +44 (0)1453 796402  
Email: NAME@gaucheralliance.org  
Website: www.gaucheralliance.org

***To whom it may concern***

The International Gaucher Alliance is an umbrella organization with more than 50 members all around the world. IGA represents the interest of the Gaucher Community.

Although the most common Lysosomal Storage Disorder Gaucher Disease is very rare with an incidence of about 1 in 100,000 live births. Patient Groups have worked closely together to share their experiences and to facilitate access to treatment. Scientists and Clinicians throughout the world increasingly collaborate both in research and in the development of strategies for the management of the disease.

At the moment there are 5 available therapies for Gaucher disease, three of them are Enzyme replacement therapy (ERT) and two are substrate reduction therapy.

In these challenging times we have been regularly communicating with our members and the clinical community and the COVID 19 situation has brought many difficulties and challenges to Gaucher patients and their families.

One of the most challenges issues is being able to receive their ERT infusions, as this means they need to go to the hospital every 2 weeks which is not possible due to hospitals treating COVID-19 patients and other emergency care, thus patients are missing their infusions.

In many countries' patients receive their infusions at home through a supported service by their local hospitals and treating Gaucher doctor.

- ERT is administered by peripheral intravenous infusion. It has been available on the market for over 20 years, and it is usually well tolerated, and the methods of administration are well known.

- ERT is usually administered in hospitals, but ERT is registered also for home therapy as to support better quality of life of patients with Gaucher disease and their families.

In many EU countries, after agreement with doctors' patients can manage treatment at home, sometimes with medical assistance and sometimes on their own.

- There are many EU countries that already authorized intravenous home therapy which showed increased comfort, better compliance and reduced costs for the health system.

- In the current pandemic context, during a recent webinar organized by the European Working Group on Gaucher Disease (EWGGD) the members who are leading Gaucher specialists recommendations to provide home treatment wherever it is possible. Given the evolution of the pandemic and the burdens linked to administration under medical supervision, we agree that fast action is required to go forward and plan home therapy for Gaucher patients.

IGA is sharing this recommendation as to support patients, families and patient organizations to continue with their therapy in this time of COVID 19 situation. We hope you will take this into consideration. We are available to share best practice regarding home therapy and answer additional questions.

***Sincerely,***

**Vesna Aleksovsk, IGA chair**

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