

Statement on Home Therapy

Home therapy with enzyme replacement therapy for Gaucher disease: a short statement from the European Working Group on Gaucher Disease (EWGGD)

Since the availability of enzyme replacement therapy for Gaucher disease in 1990, many patients have received their treatment at home. In several countries in the EU as well as in Israel, home therapy has been implemented from early in the patient's treatment. An inventory of experiences in the Netherlands, the UK and France some years ago showed that most patients preferred to have their treatment at home. After one or more training sessions, many patients are either self-supporting, or administer their medication with assistance of their spouse or a family member. Some prefer support by a homecare team, mostly because they need assistance with venous access. Imiglucerase as well as velaglucerase is well tolerated with no severe infusion related reactions based upon experiences over the last 30 years for imiglucerase and 10 years for velaglucerase. This indicates a very high safety profile for both of these authorized enzymes. In Israel, home therapy supported by homecare team has also been used safely for patients receiving taliglucerase.

During this challenging period of the COVID-19 pandemic, we learned that in some countries treatments were interrupted, because hospital infusions were cancelled. In line with international recommendations (Mistry et al 2020), we support the view that missing a couple of infusions will in most cases not lead to immediate deterioration of disease. However, long interruptions should be avoided especially in children and in such cases home therapy may be a solution to continue treatment. Some home infusion companies have immediately adopted safe measures to deliver homecare to continue or transfer treatment to homes. The EWGGD is available to offer support for changing practice in countries where home therapy is not yet approved. In addition, it is important to know that so far as our experience is available there is no indication that patients with Gaucher disease are specifically vulnerable to become infected with SARS-CoV-2 or will experience a more severe disease course.

Reference: Mistry P et al. Gaucher disease and SARS-CoV-2 infection: Emerging management challenges. *Mol Genet Metab.* 2020 Jul; 130(3): 164–169. Published online 2020 May 11.