GUIDELINES ON HOME THERAPY IN GAUCHER DISEASE

INTRODUCTION:
Home-based enzyme replacement therapy (ERT) for Gaucher disease (GD) is well established and gained relevance during COVID-19. To support its safe use, the International Gaucher Alliance (IGA) and the International Working Group on Gaucher Disease (IWGGD) developed multidisciplinary guidelines for home infusions.

METHODOLOGY:
- **Narrative literature review**
- **2022 international survey (IGA member organizations)**
- **Focus: safety, feasibility, outcomes, and implementation.**

RESULTS:
- **Home-based ERT is safe and feasible**
- **High patient satisfaction and improved quality of life**
- **Reduced hospital visits and resource use**
- **Infusion reactions are rare and mainly occur at treatment initiation**

IGA Website | IWGGD Website | Literature review of home therapy | Contact information

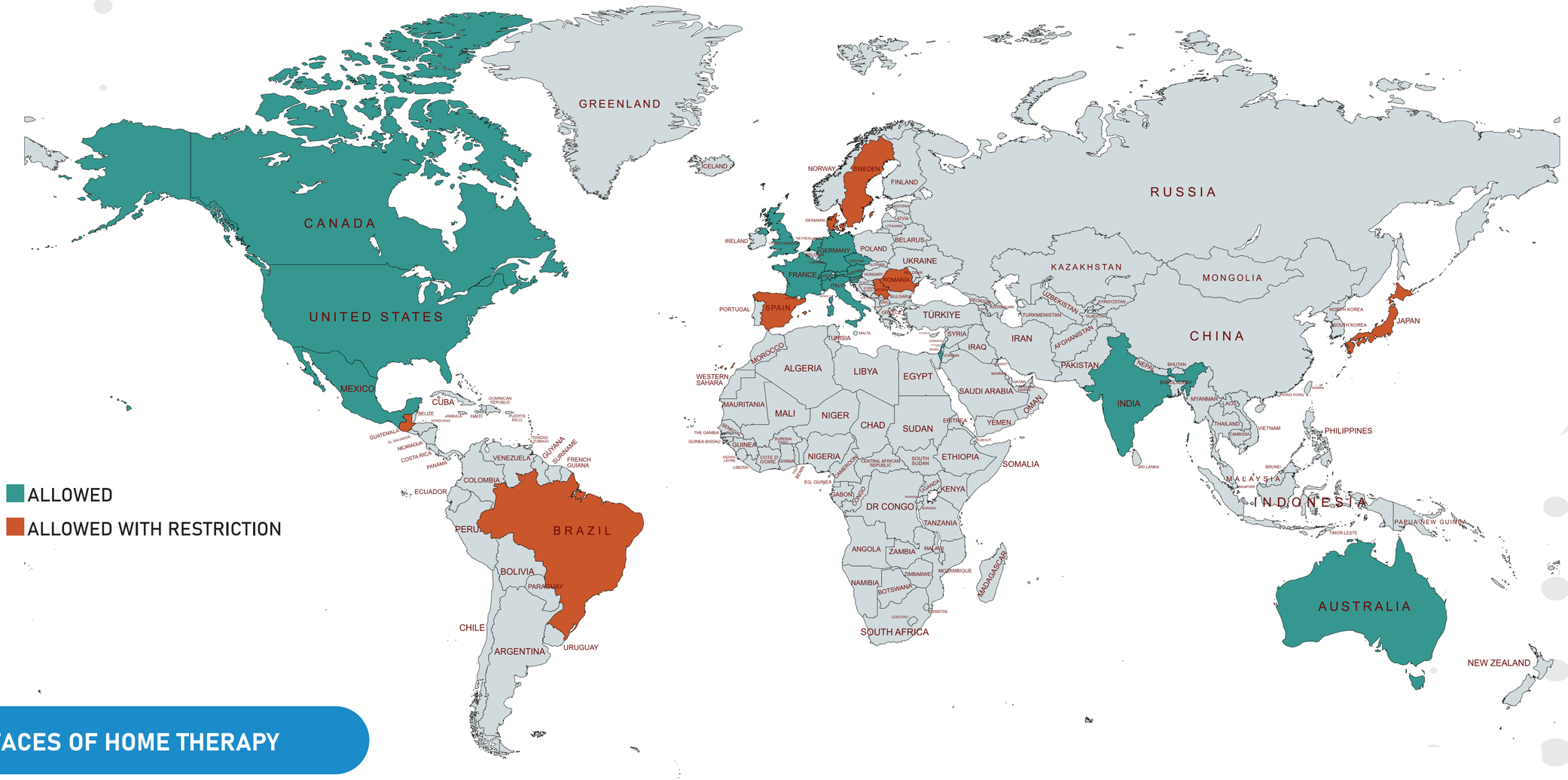
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Survey findings

- **Available in 62.5% of countries**
- **Benefits: less travel, shorter waiting times, greater flexibility, better continuity of care**
- **Economic advantages for patients and healthcare systems**

BARRIERS:
- **Regulatory and legislative limitations**
- **Logistical challenges (delivery, storage)**
- **Confidentiality concerns**

DISCUSSION:
- **Patient-centered approach with clear clinical and economic benefits**
- **Unequal access across countries persists**
- **Need for guidelines, best-practice dissemination, and advocacy**
- **Multidisciplinary collaboration is essential**



SOME OF THE FACES OF HOME THERAPY

















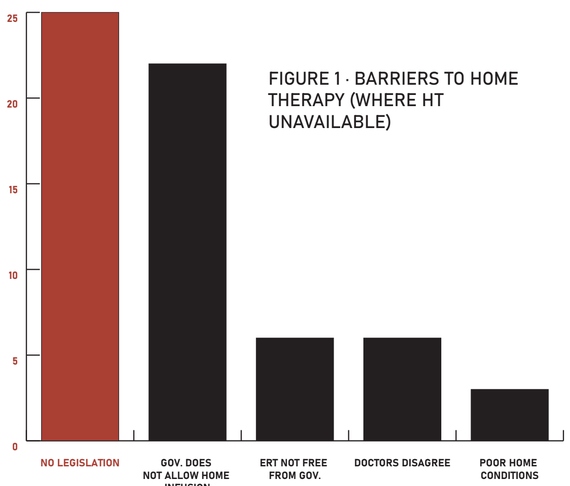


FIGURE 1 · BARRIERS TO HOME THERAPY (WHERE HT UNAVAILABLE)

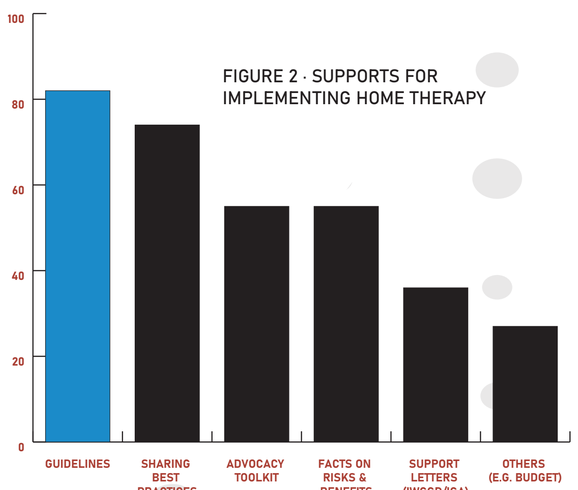
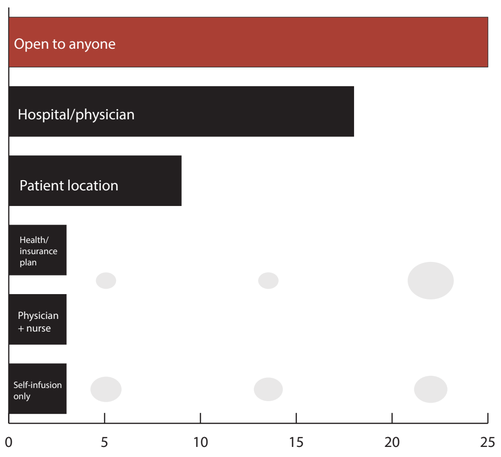


FIGURE 2 · SUPPORTS FOR IMPLEMENTING HOME THERAPY

Survey findings

FIGURE 3 · FACTORS DETERMINING ACCESS TO HOME THERAPY



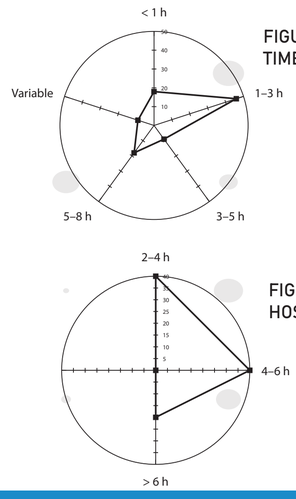
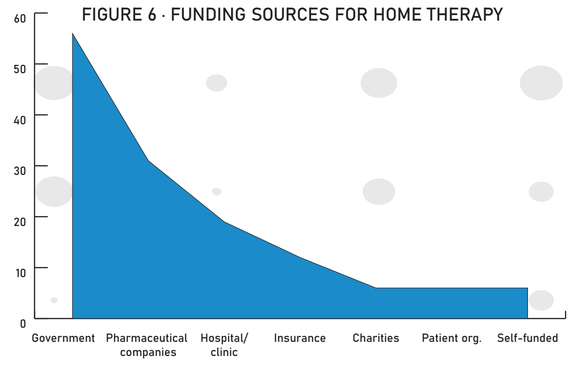


FIGURE 4 · TRAVEL TIME TO HOSPITAL

FIGURE 5 - TIME SPENT IN HOSPITAL

FIGURE 6 · FUNDING SOURCES FOR HOME THERAPY



CONCLUSION:

This work supports the development of an educational leaflet to promote safe and effective home infusions worldwide.