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# Genetic Testing and Gene Therapy Resources for Providers and Patients<sup>1</sup>

Full abbreviations, accreditation, and disclosure information available at [PeerView.com/GWQ40](https://www.peerview.com/GWQ40)

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## Genetic Testing and Gene Therapy Resources for Patients With Gaucher Disease

**Gaucher disease is a** rare genetic disorder caused by a deficiency in the lysosomal enzyme glucocerebrosidase, leading to harmful buildup of fatty substances in cells and tissues, affecting organs like the spleen, liver, and bones

**Gene therapy is a** treatment that introduces a healthy copy of the *GBA1* gene into the body to help produce the missing or faulty enzyme



**Why consider gene therapy?** It offers a potential for a one-time, long-lasting treatment option while other available treatments require lifelong use

**Who can get gene therapy?** Gene therapy is still being studied in clinical trials; if interested, you may be eligible to participate in a clinical trial depending on age, disease type, and overall health



Peer community support, education, and resources on Gaucher disease from  
**Gaucher Community Alliance (GCA)**



Gene therapy for Gaucher disease and clinical trial information provided by  
**American Society of Gene and Cell Therapy (ASGCT)**



Genetic screening and counseling information provided by  
**National Gaucher Foundation (NCF)**





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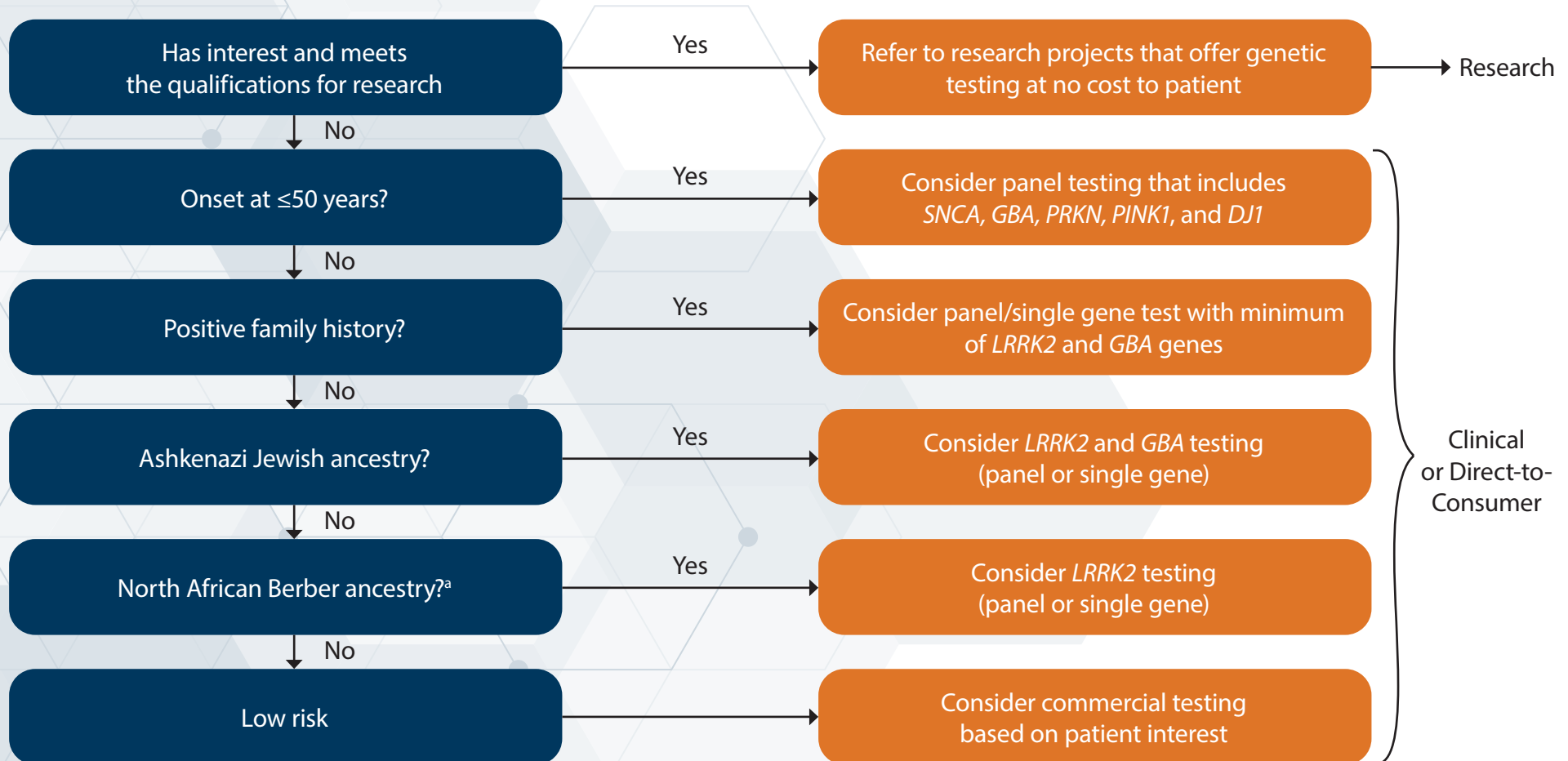
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## Proposed Pathway for Genetic Testing in Patients With Parkinson's Disease



<sup>a</sup> There are additional founder effects reported in the literature; recommendations for specific testing will depend on the population.  
1. Cook L et al. *Neurol Clin Pract*. 2021;11:69-77.