



GLOBAL DISEASE REGISTRY FOR NEURONOPATHIC GD

The IGA is an international umbrella group representing the interest of Gaucher patients and those of non-for-profit Gaucher patient groups as well as rare disease groups throughout the world.

AUTHORS

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PATIENT INVOLVEMENT

10 volunteers via focus group and in-depth interviews
On-line Delphi with 23 responders from 13 countries.
Important themes and outcomes identified for further research and validation.



Neuronopathic Gaucher Disease (nGD) has a high unmet need, but with an increasing number of pharma companies now developing treatment options, it brings hope to many patients and families.

A collaborative disease registry would offer important insight for understanding natural history, validating new outcomes and support clinical trial designs, recruitment, as well as regulatory and reimbursement reviews

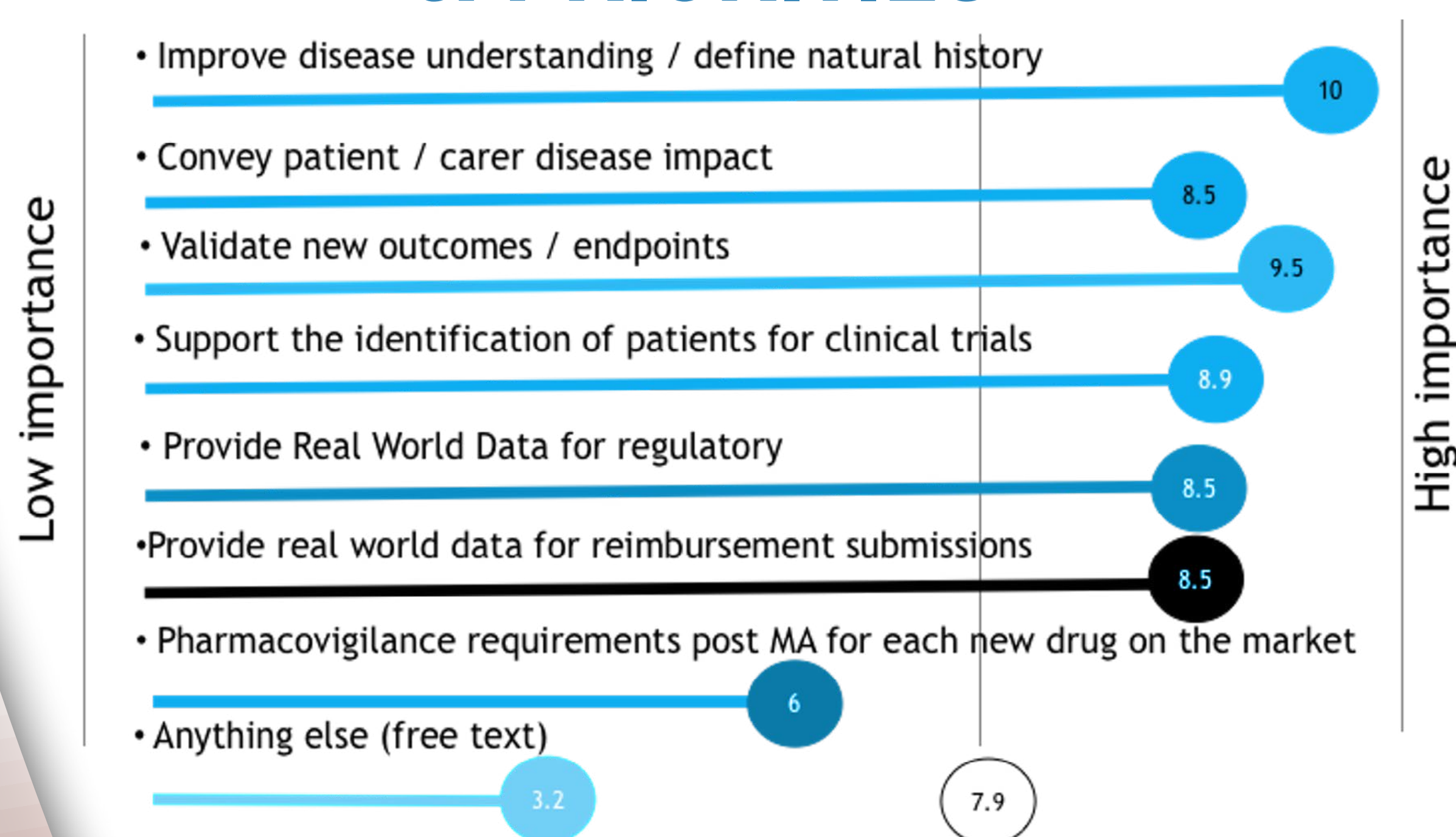
KEY OPINION LEADERS

1 MEETING
5 KOLs from 3 countries
On-line Delphi
Input from 12 KOLs in 5 countries
132 clinical data fields identified

TECHNICAL REQUIREMENTS

Technical architecture of the ideal solutions were defined
Compliance and regulation a mandatory component (e.g. GDPR, GCP, Part 11)

PHARMA REQUIREMENTS & PRIORITIES



PHARMA PARTNERS

The IGA would like to thank the four pharmaceutical companies that invested in the development of phase 1 through their participation in the pharma group element of the project and also for their financial support through a unrestricted grant.

