

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.

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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)



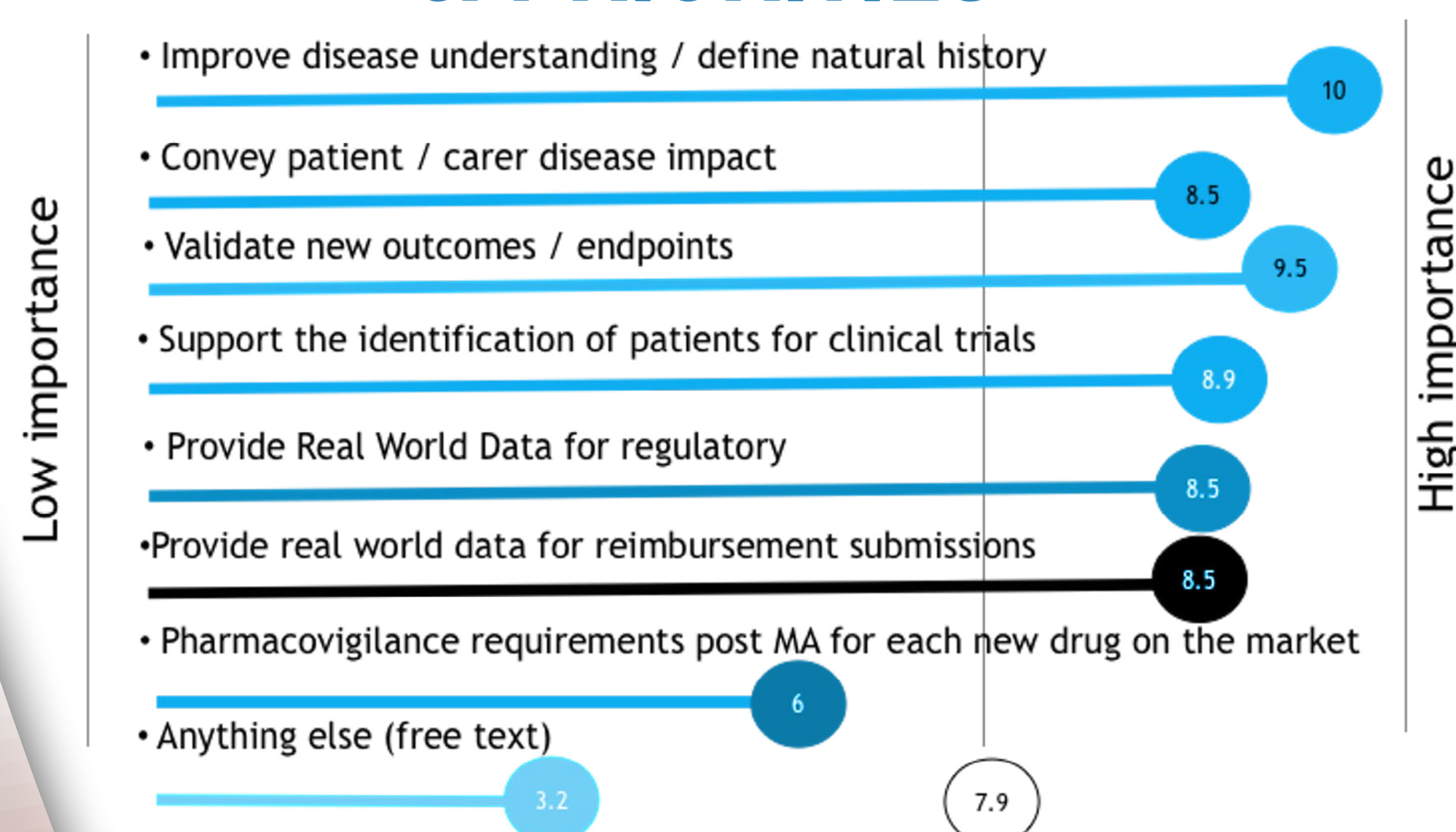
The IGA acted as a lead instigator, bringing together all the partners with funding from the pharma partners and support from Aparito to explore patient, KOL, and pharma needs and requirements.

Recommendation for next steps:

There was high level of enthusiasm to support the initiative by all stakeholders.

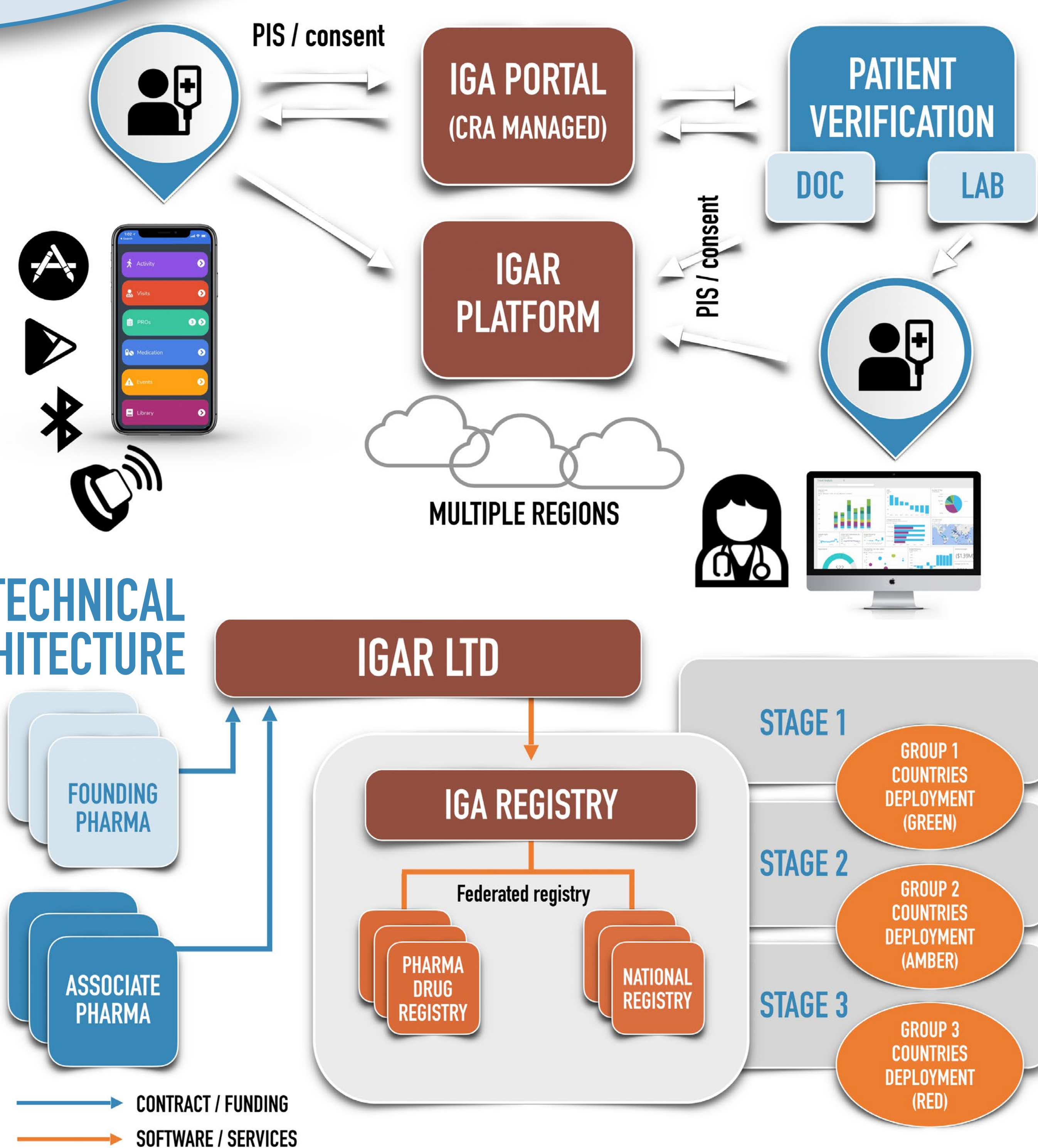
- SUSTAINABLE BUSINESS MODEL THROUGH A PARTNERSHIP AGREEMENT
 - COLLABORATIVE, GLOBAL FEDERATED REGISTRY (IGAR)
 - CLEAR AND TRANSPARENT GOVERNANCE
 - SEEK EMA/FDA BUY-IN AND APPROVALS
 - STAGGER OVER THREE STAGES TO BUILD TRUST
 - INCORPORATE FAIR DATA PRINCIPLES

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.



Working in collaboration with



Pfizer
Rare Disease



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