THEME 3: SHARE, CARE, RARE: TRANSFORMING CARE FOR RARE DISEASES BY 2030

POSTER 116



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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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.

cidal socialising

end-of-life Anxiety

pressure su

Resilience Coping self-help

Socialclaustrophobia
Difficult restricted-services
Milestones cutting-hair
Life-stage-expectations

importance

Low

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

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.

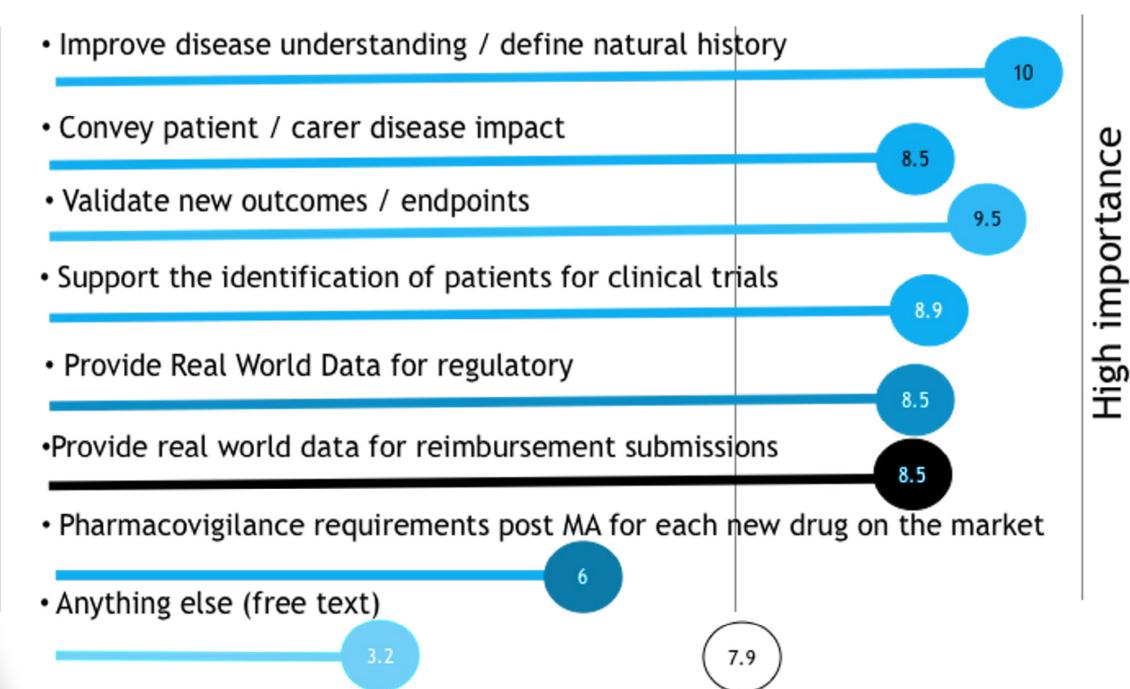
- SUSTAINABLE BUSINESS MODEL THROUGH A PARTNERSHIP AGREEMENT
 - COLLABORATIVE, GLOBAL FEDERATED REGISTRY (IGAR)
 - CLEAR AND TRANSPARENT GOVERNANCE
 - SEEK EMA/FDA BUY-IN AND APPROVALS
 - STAGGER OVER THREE STAGES TO BUILD TRUST
 - INCORPORATE FAIR DATA PRINCIPLES

TECHNICAL REQUIREMENTS

Technical architecture of the ideal solutions were defined

Compliance and regulation a mandatory component (e.g. GDPR, GCP, Part 11)

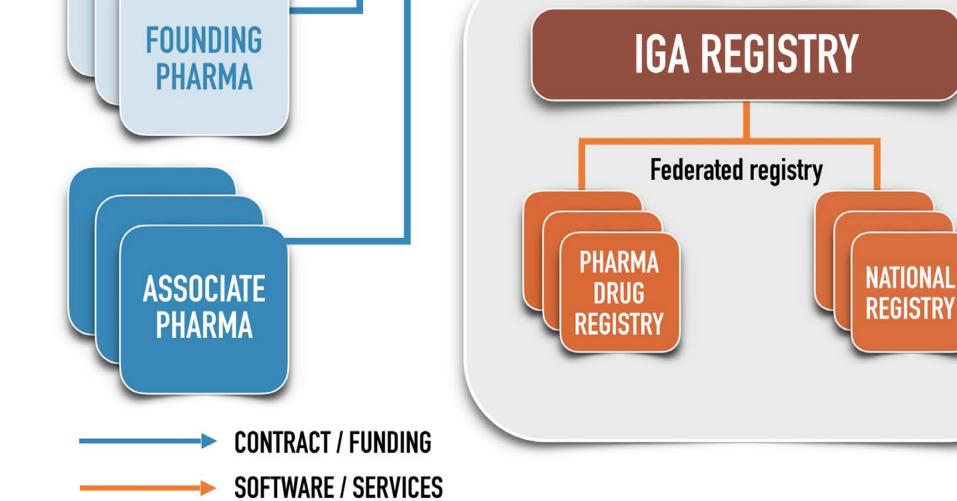






PHARMA PARTNERS

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.



PIS / consent

Prevail

Working in collaboration with aparito KANTAR







Pfizer





GROUP 1

COUNTRIES

DEPLOYMENT

(GREEN)

GROUP 2

COUNTRIES

DEPLOYMENT

(AMBER)

GROUP 3

COUNTRIES

DEPLOYMENT

STAGE 2

STAGE 3