

Research Priorities for Liver Glycogen Storage Disease: An International Priority Setting Partnership with the James Lind Alliance

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For which topic were research priorities identified?

liver glycogen storage disease

In which location was the research priority setting conducted?

international

Why was it conducted at all?

Liver glycogen storage diseases (GSD) are ultra-rare diseases, among the oldest known inborn errors of metabolism described in literature, and classified according to the protein deficiency and the organ distribution. The international GSD community has a longstanding tradition of involving patient representatives in directing healthcare and research. However, there are discrepancies between questions considered relevant by patients, carers, and healthcare professionals, and the research performed in rare diseases.

What was the objective?

to address research priorities of direct relevance and potential benefit to liver glycogen storage disease patients, carers, and the treating healthcare providers

What was the outcome?

a ranking list of 11 research questions

How long did the research prioritization take?

November 2016 - May 2019

Which methods were used to identify research priorities?

JLA method

How were the priorities for research identified exactly?

Step 1: setting up PSP: steering group established, stakeholders and partner organizations identified. Step 2: gathering uncertainties: survey asking: What are your questions on the care and/or management of liver Glycogen Storage Disease?, 1388 questions submitted. Step 3: data processing: responses categorized, subcategorized, out-of-scope eliminated, summary questions formulated, check against evidence, resulting in 72 summary questions. Step 4: interim ranking: survey asking participants to choose their top 10 research questions on the care and/or management of liver GSD, top 10 questions for each stakeholder group identified, resulting in shortlist of 22 summary questions taken forward to workshop. Step 5: final prioritization: workshop with nominal group technique: small group discussions and small group rankings, followed by plenary discussion

Which stakeholders took part?

Glycogen storage disease patients, carers, and the treating healthcare providers. Survey: 763 participants. Interim ranking: 563 participants. Workshop: 22 participants.

How were stakeholders recruited?

Survey was distributed to patients, carers, and healthcare professionals by multiple partner organizations, such as patient organizations, professional networks, and individual patients, carers, and healthcare professionals. Via an open call on the IGSDPSP website, social media, and by steering group members, people with liver GSD, carers, and healthcare professionals were suggested as participants for the final priority setting workshop at the Selbsthilfegruppe Glykogenose Deutschland e.V. in Duderstadt (Germany).

Were stakeholders actively involved or did they just participate?

Stakeholders not only participated but were also actively involved in the research prioritization process: They were part of a steering group. The steering group consisted of 14 members from 12 countries of whom some represented more than one stakeholder group: 10 carers or patients, 9 healthcare professionals (7 physicians, 1 nurse, 1 dietician), and 8 representatives of patient organizations. The members oversaw the process and were involved in all steps.