Background

- Although safety information on recombinant human growth hormone (rhGH) therapy has now been collected for several years, there are still existing gaps in our knowledge. With the introduction of new indications and novel forms of rhGH, the need for a global registry has never been greater.
- Previously, assessment of safety and efficacy of rhGH therapy has primarily relied on the use of pharmaceutical industry-led registries for post-regulatory approval studies (PAS), linked national datasets in individual countries or by pooling these linked national datasets from individual countries; each with their own limitations.
- A major common limitation of all these approaches, however, has been the lack of standardisation of data that were collected, resulting in potential outcome bias.
- With the introduction of new indications and novel forms of rhGH, the need for a global registry has never been greater.

Methods

- This exercise was undertaken through the GH Scientific Study Group (SSG) and the GH Scientific Studies Committee in GloBE-Reg, a common registry platform that collects real world data for long-term safety and effectiveness studies of drugs.
- 12 clinical experts from 7 international endocrine organisations, representatives from 2 pharmaceutical companies with previous GH registry expertise and 2 patient advocates collaborated to develop this recommendation.
- A comprehensive list of data fields routinely collected by each of the clinical and industry experts was compiled
- Each member was asked to determine the:
  1. Importance of the data field, and
  2. Ease of data collection.
- Data fields that achieved 70% consensus in terms of importance qualified for the minimum dataset, provided <50% deemed the item difficult to collect.

Objective

- To identify the minimum dataset (MDS) that could be measured in a routine clinical setting across the world

Results

- Of the 246 items initially compiled, only 219 were subjected to grading with a final MDS recommendation of 43 items; 20 to be completed once, 14 every 6 months and 9 every 12 months.
- Several anomalies to the MDS rule were identified and examined on their individual merits with 4 items added into the MDS

Conclusion

- This exercise performed through the GloBE-Reg initiative provides a recommendation of the minimum dataset requirement, collected through real-world data, for the monitoring of safety and effectiveness of rhGH in children with GHD, to minimise burden and improve quality of data collection.