European Achondroplasia Forum Assessing Outcomes in a New Era of Achondroplasia Treatment

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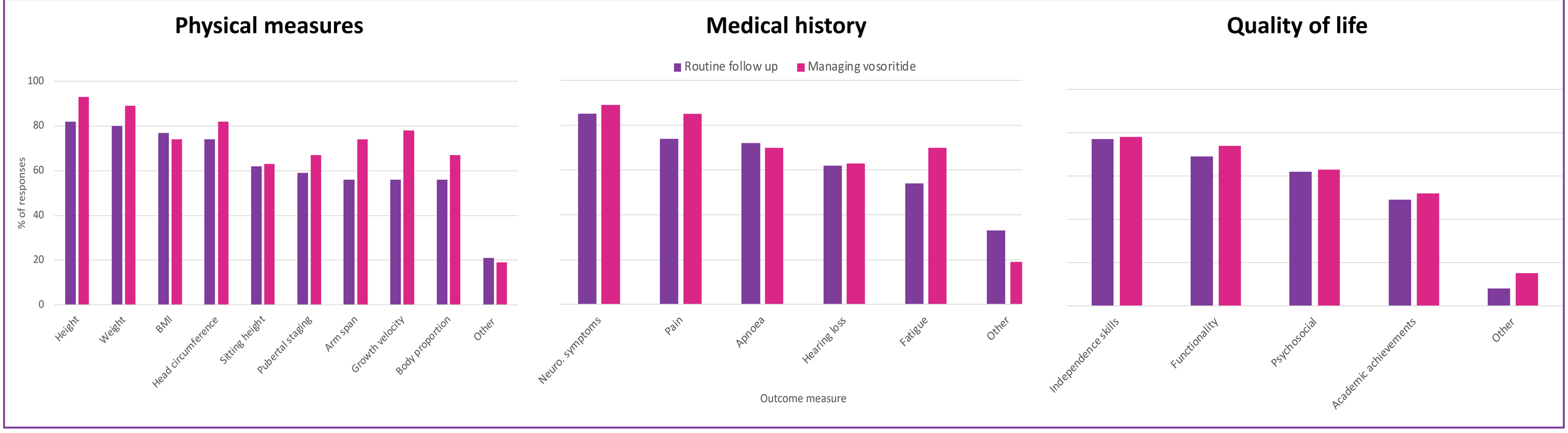
Background

- Achondroplasia requires lifelong multidisciplinary care
- Vosoritide, which targets the FGFR3-signalling pathway, is the first commercially available therapy to treat achondroplasia; several other medical therapies are also in development
- The European Achondroplasia Forum (EAF) reviewed existing health surveillance measures to determine if
  1. They still apply in clinical practice and in assessing impact of medical therapy
  2. New/updated measures are needed

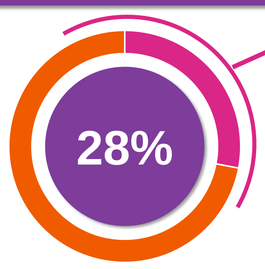
Methods

- An online questionnaire assessed health status monitoring, both in routine clinical practice and in determining response to vosoritide
  - 50 respondents representing a range of specialities from 14 countries across Europe, Latin America, the Middle East, and the USA completed the survey
- Results were discussed during an EAF workshop, held in April 2024
  - The workshop was joined by 63 attendees (38 healthcare professionals, 19 industry representative, three patient advocacy group representatives)

Responses from the EAF survey: In current practice outcomes monitored as part of routine follow-up reflect those in children receiving vosoritide

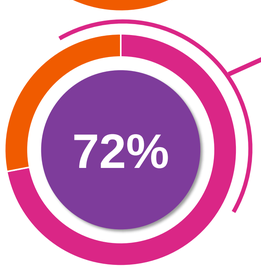


Are outcome measures still fit for purpose?



Of respondents felt that current measures **did not accurately capture outcomes in routine practice**

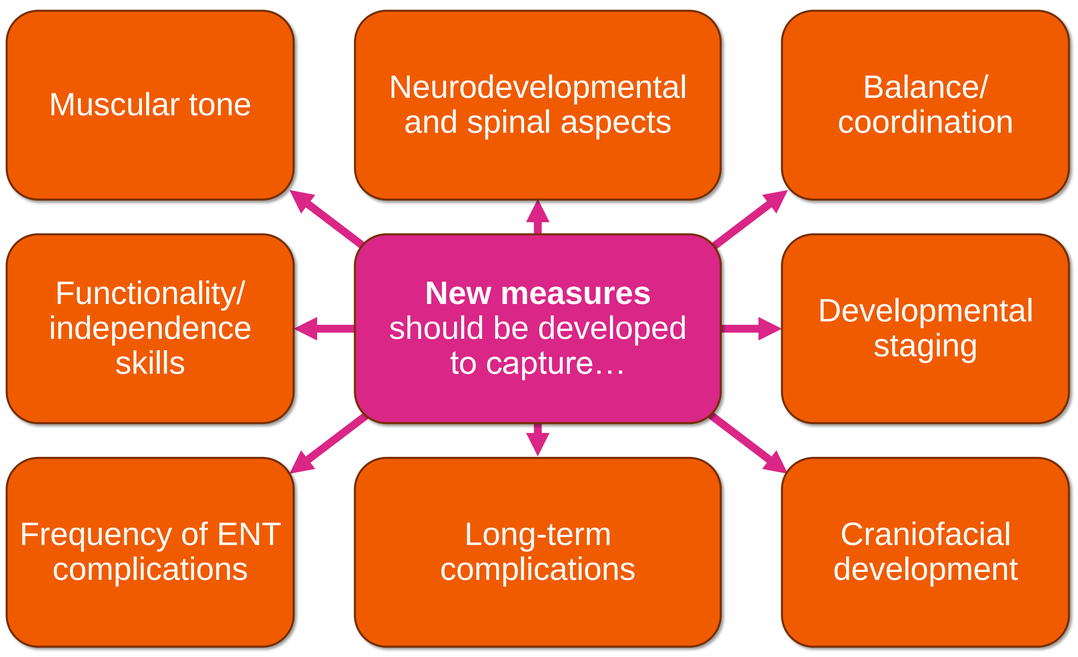
Of respondents managing children receiving vosoritide felt that **current measures were not accurately capturing outcomes after treatment**



Of respondents were **not collecting data in a registry**

Does the current system of capturing outcomes work effectively?

With the advent of medical therapies, what is needed for the future?



Current measures do not **exist** or do not **capture** necessary data

- Limitations in resources/funding lead to **variability in outcomes** measured and data captured
- **Quality of life** is particularly challenging; some centres do not attempt to assess this due to time constraints

Conclusions

- **Collecting accurate, standardised data is essential** to understanding the daily impact of achondroplasia, and determining the effect of any intervention
- **Outcome measures** exist but are **not consistently and accurately used** in routine clinical practice or following up children receiving vosoritide
- With the advent of innovative therapies, this is an opportune moment to ensure outcome measures align with evolving practice
- **Agreement on a standardised core set of outcomes and, a simplified, time efficient way of collecting quality of life data is needed**

EAF
European Achondroplasia Forum

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