Use of growth hormone therapy in short patients born small for gestational age: data from real-life French clinical practice

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Baseline characteristics

Of the 1406 registered patients, every fifth child was randomly selected for the long-term follow-up as a representative subpopulation (n=291), for efficacy and safety analyses. The baseline characteristics between these two groups were similar (Table 1).

Table 1  Baseline characteristics

<table>
<thead>
<tr>
<th>Variable</th>
<th>Overall (n=1406)</th>
<th>Subpopulation (n=291)</th>
<th>p-value</th>
</tr>
</thead>
<tbody>
<tr>
<td>Age at study inclusion, years</td>
<td>2.0 (0.7; 3.3)</td>
<td>1.9 (0.7; 3.2)</td>
<td>0.25</td>
</tr>
<tr>
<td>Sex</td>
<td>Male (65.9%)</td>
<td>Male (61.1%)</td>
<td>0.33</td>
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<tr>
<td>Diabetes</td>
<td>2.9%</td>
<td>3.1%</td>
<td>0.59</td>
</tr>
</tbody>
</table>

RESULTS

Effectiveness

- Normal height standard deviation score (HSDS) was reached by 66.3% of patients at last visit. The proportion of patients reaching normal HSDS was similar between previously treated and treatment-naive patients (Figure 2).

- Among the 24.7% of patients who achieved final adult height at last visit, 66.7% reached normal HSDS.

Safety

- There were 287 adverse events (AEs) reported in 149 (51.2%) patients (Table 3).

- The most frequent AEs were increased insulin-like growth factor 1 (IGF-1; 17.2%) headache (9.3%), and arthralgia (4.5%), with most (n=71/100) of these events reported in treatment-naive patients.

- Sixteen (5.5%) of the 291 patients discontinued treatment prematurely, most commonly due to increased IGF-1 (n=4).

- Five AEs of special interest were reported, none of which were considered related to GH treatment.

- Two tumours or tumour-like lesions: Malignant nephroblastoma with a fatal outcome.

- One cardiovascular event: Tricuspid valve incompetence.

- One cerebrovascular event: Ventriculo-cardiac shunt due to hydrocephalus.

CONCLUSIONS

- GH therapy was effective in most short children born SGA.

- No new safety concerns were observed with use of GH therapy.

- The likelihood of achieving normal HSDS with GH therapy increased with greater baseline HSDS, younger age at start of treatment, longer duration of GH treatment and absence of a chronic disease; reinforcing the importance of early identification and treatment of short patients born SGA.

REFERENCES