

Summit Meeting | Wednesday 13 November, 2024

Global Access to Therapies for People with Prader-Willi Syndrome: Understanding the Challenges and Seeking Solutions



A global investigation of the approval, availability, and
affordability of growth hormone treatment for children and
adults with the rare neurodevelopmental condition,
Prader-Willi Syndrome

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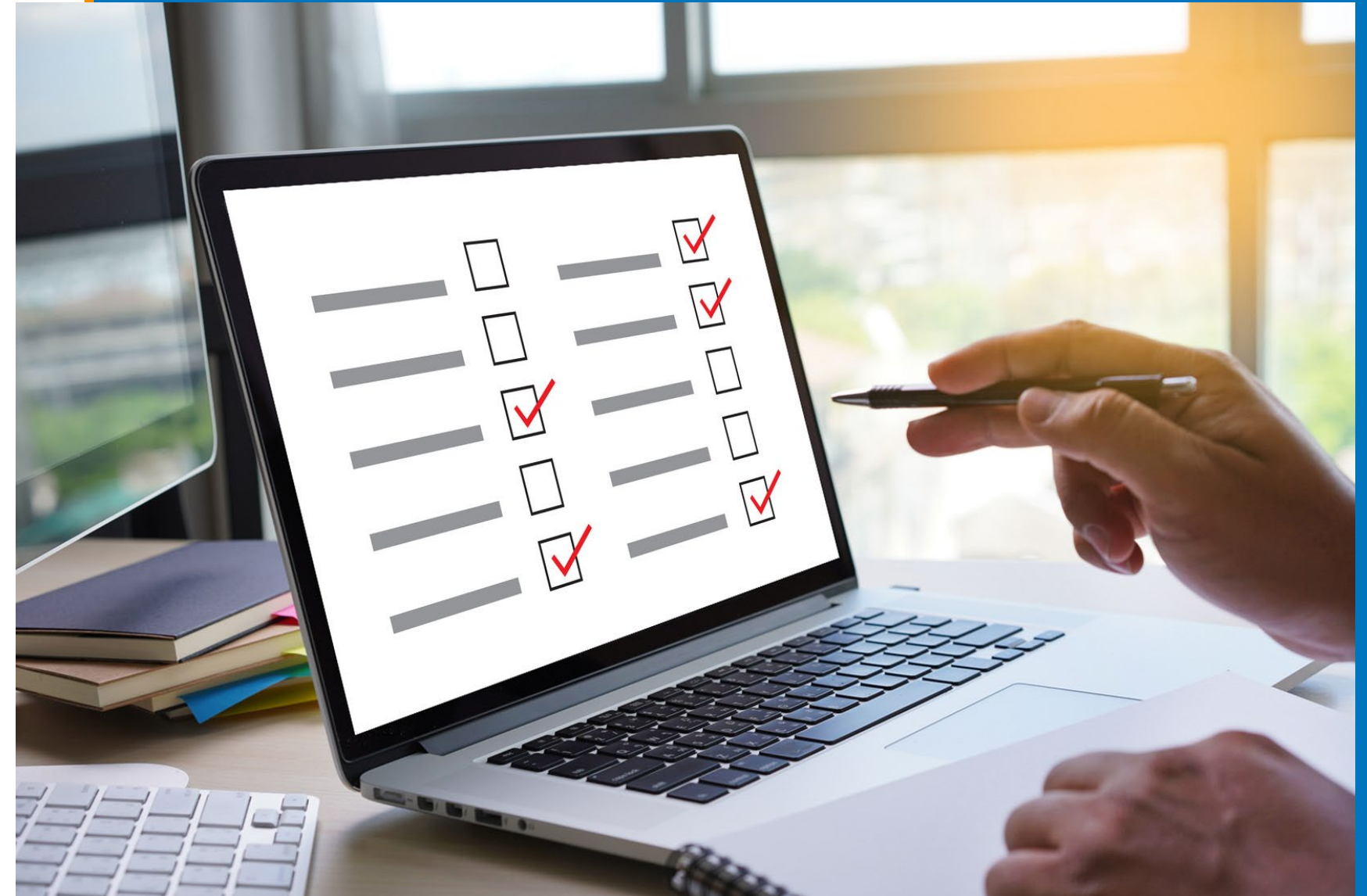
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IPWSO GLOBAL GH STUDY

Aims:

To establish the extent to which globally GH has been approved for children and adults with PWS, and whether or not it is available and affordable.

Presentation of preliminary findings, further analysis still to take place.



METHODS

- Development of internet-based survey
- Piloting of survey by parents and a medical professional
- Distributed globally by personal invitation to a parent and a medical professional in each country
- Responders asked to consider the wider national circumstances, not just individual experience
- Available in English, Spanish, Arabic, and French
- Completed survey returned to SC at IPWSO
- Anonymised completed questionnaires send to JW for analysis
- Also information asked about PWS in general and local services
- Information on national economic status and population size obtained

RESPONDENTS

127 respondents from 80 countries

| Number (countries) | Economic status (Income) | Population (Range) |
|-----------------------|-----------------------------|-----------------------|
| 39 (49%) | High | 3 to 365 million |
| 19 (24%) | Medium high | 1 to 1419 million |
| 18 (22%) | Medium low | 6 to 1451 million |
| 4 (3%) | Low | 3 to 132 million |

APPROVAL SYSTEM/GH APPROVAL

Percentage of countries in each socio-economic group

| | | High N=39 | Medium high N=19 | Medium low N=18 | Low N=4 | Total N=67 |
|----------|------------------|--------------|------------------------|-----------------------|------------|---------------|
| APPROVAL | System approval | 35 (90%) | 16 (84%) | 14 (78%) | 2 (50%) | |
| | No system | 3 | 3 | 1 | 2 | 9 |
| | Don't know | 1 | 0 | 3 | 0 | 4 |
| | | | | | | |
| | | | | | | |
| APPROVED | Child only | 22 (56%) | 7 (37%) | 3 (21%) | 0 | 32 |
| FOR | Child & adult | 15 (38%) | 4 (21%) | 10 (55%) | 1 (25%) | 30 |
| | No PWS | 0 | 6 | 2 | 2 | 10 |
| | Don't know | 2 | 2 | 3 | 1 | 8 |
| | Total (approved) | 37 (95%) | 11 (58%) | 13 (72%) | 1 (25%) | |

ESTIMATED EXTENT OF GH USE BY CHILDREN WITH PWS IN 75 COUNTRIES DIVIDED ACCORDING TO SOCIO-ECONOMIC STATUS

| | | High N=38 | Medium High N=17 | Medium Low N=17 | Low N=3 | Total N=75 |
|-------|--------------------|--------------|------------------------|-----------------------|------------|---------------|
| CHILD | All are using | 14 (37%) | 0 | 0 | 0 | 14 |
| USAGE | Most are using | 17 (45%) | 5 (29%) | 2 (12%) | 1 | 25 |
| | About half using | 1 (3%) | 1 (6%) | 2 (12%) | 0 | 4 |
| | Most are not using | 3 (8%) | 5 (29%) | 8 (47%) | 2 | 18 |
| | None are using | 0 | 1 (6%) | 1 (6%) | 0 | 2 |
| | Not available | 3 (8%) | 5 (29%) | 4 (24%) | 0 | 12 |

Extensive use of GH treatment in children with PWS predominately dependent on socio-economic status of the country

ESTIMATED EXTENT OF GH USE FOR ADULTS WITH PWS IN 69 COUNTRIES DIVIDED ACCORDING TO SOCIO-ECONOMIC STATUS

| | | High N=35 | Medium High N= 14 | Medium Low N=17 | Low N=3 | Total N=69 |
|-------|--------------------|----------------------|----------------------------------|--------------------------------|--------------------|-----------------------|
| ADULT | All are using | 0 | 0 | 1 (6%) | 0 | 1 (1%) |
| USAGE | Most are using | 5 (14%) | 1 (7%) | 0 | 0 | 6 (9%) |
| | About half using | 6 (17%) | 0 | 0 | 0 | 6 (9%) |
| | Most are not using | 17 (49%) | 7 (50%) | 7 (41%) | 1 | 32 (46%) |
| | None are using | 1 (3%) | 2 (14%) | 3 (18%) | 0 | 6 (9%) |
| | Don't know | 6 (17%) | 4 (29%) | 6 (35%) | 2 | 18 (26%) |

Regardless of socio-economic status less than 50% of adults with PWS are estimated to be receiving GH in any given country

AVAILABILITY OF GH AND ACCESS TO PWS DIAGNOSIS

| | | High N=38 | Medium high N=17 | Medium low N=17 | Low N=3 | Total N=75 |
|-----------|----------------------|--------------|------------------------|-----------------------|------------|---------------|
| WHERE | All parts country | 30 (79%) | 8 (47%) | 5 (29%) | 0 | 43 (57%) |
| AVAILABLE | Some parts only | 3 (8%) | 7 (41%) | 9 (52%) | 0 | 19 (25%) |
| | Don't know | 5 (13%) | 2 (12%) | 3 (18%) | 3 | 13 (17%) |
| | | | | | | |
| | | N=39 | N=19 | N=18 | N=4 | N=80 |
| DIAGNOSIS | Across country | 22 (56%) | 4 (21%) | 1 (6%) | 0 | 27 (34%) |
| OF PWS | Certain centres | 17 (44%) | 12 (63%) | 9 (50%) | 1 (25%) | 39 (49%) |
| | Not available | 0 | 3 (16%) | 8 (44%) | 3 (75%) | 14 (18%) |

ANSWERS FROM RESPONDENTS IN EACH COUNTRY TO THE QUESTION – WHO PRESCRIBES GH?

| | Endocrinologist | Any doctor | Total |
|---------------------------|-----------------|------------|---------------------|
| Agreement of responses | 16 | 1 | 17 |
| Disagreement of responses | | | 3 |
| Single response only | 37 | 4 | 41 |
| Missing | | | 12 |
| Total | 53 | 5 | 73 (58+3+12) |

Agreement of responses: both respondents from the same country agreed

RESPONDENTS' ANSWERS TO THE QUESTION: IS EVIDENCE OF GH DEFICIENCY NEEDED TO PRESCRIBE GH?

| | Lab Evidence of GHD | Diagnosis PWS is sufficient | Don't know | Other | Total |
|--------------|------------------------|-----------------------------------|------------|-------|-------|
| Professional | 12 | 46 | 1 | 2 | 61 |
| Family | 16 | 33 | 2 | 6 | 57 |
| Total | 28 | 79 | 3 | 8 | 118 |

Comments:

practice varies across the country, clinicians ignorant about PWS, difference between official conditions and actual experience in practice

ANSWERS FROM RESPONDENTS IN EACH COUNTRY TO THE QUESTION – WHO PAYS FOR GH?

| | Free from health service | Subsidised by health service | Full or part from health insurance | Family pays | Total |
|---------------------------|--------------------------|------------------------------|------------------------------------|-------------|--------------|
| Agreement of responses | 17 | 2 | 1 | 2 | 22 |
| Disagreement of responses | | | | | 12 |
| Single response only | 14 | 4 | 6 | 17 | 41 |
| Missing | | | | | 2 |
| Total | 31 | 6 | 7 | 19 | 77 (63+12+2) |

Comments:

different funding systems could operate in the same country, depends on the age of the person with PWS, on parental employment or family income

RESPONDENTS' ANSWERS TO THE QUESTION:
ARE SLEEP STUDIES UNDERTAKEN BEFORE PRESCRIBING GH?

| | Yes | No | Don't know | Other | Total |
|--------------|-----|----|------------|-------|-------|
| Professional | 36 | 15 | 3 | 7 | 61 |
| Family | 40 | 6 | 6 | 6 | 58 |
| Total | 76 | 21 | 9 | 13 | 119 |

THEME: IGNORANCE ABOUT PWS

“
Diagnostic
methods not
available
”

“
Still not enough
doctors aware of the
syndrome
”

“
Just considered to be a child
with intellectual disabilities
”

“
They are misdiagnosed as
a child with cerebral palsy
”

THEME: AVAILABILITY OF GROWTH HORMONE

“
GH is unavailable
and over-priced
”

“
GH is available but only
given to those diagnosed
as having GH deficiency
”

“
Availability random and
very expensive
”

“
It is not available in my
country, people have to
bring it from outside
”

THEME: AFFORDABILITY OF GROWTH HORMONE

“

Available for children with PWS but adults have to pay

”

“

The price is exorbitant and unaffordable for the majority of people

”

“

Only reimbursed from the State for children with PWS

”

COMMENTS

- Further detailed analyses to be undertaken
- Access to GH treatment for children but much less so for adults with PWS
- Some countries GH approved but not for PWS
- Barriers include lack of diagnosis of PWS, limited professional awareness, variations in availability, accessibility and affordability

WHO ESSENTIAL MEDICINES LIST FOR CHILDREN

“ Essential medicines are those that satisfy the priority health care need of a population. They are selected with due regard to disease prevalence and public health relevance, evidence of efficacy and safety and comparative cost-effectiveness. They are intended to be available in functional health systems at all times, in appropriate dosage forms, of assured quality and at prices individuals and health systems can afford.

GH not included

FINALLY

Thank you to the parents and professionals in many countries who completed the questionnaire.

Questions?

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