

While the true prevalence of HPP remains unknown, estimates suggest it occurs in ~1 in 5,685 people\* –

**MANY OF WHOM REMAIN UNDIAGNOSED<sup>1-4</sup>**



Rethink HPP diagnosis by earlier recognition of hypophosphatasia signs and symptoms and the impact of persistently low ALP adjusted for age and sex<sup>5</sup>

\*Prevalence data has been calculated based on information presented in Tornero C, et al. 2020.<sup>1</sup>

ALP, alkaline phosphatase; HPP, hypophosphatasia.

**1.** Tornero C, et al. *Orphanet J Rare Dis.* 2020;15(1):51. **2.** Rockman-Greenberg C. *Pediatr Endocrinol Rev.* 2013;10(Suppl 2):380–388. **3.** NORD. Hypophosphatasia. Available at: <https://rarediseases.org/rare-diseases/hypophosphatasia/#complete-report> (Accessed February 2025). **4.** Tournis S, et al. *J Clin Med.* 2021;10(23):5676. **5.** Khan AA, et al. *Osteoporos Int.* 2024;35(3):431–438.

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**ALEXION**<sup>®</sup>  
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The estimated median

**DIAGNOSTIC  
DELAY IS  
~10 YEARS**

in adults with HPP\*<sup>1</sup>



Rethink HPP diagnosis by earlier recognition of hypophosphatasia signs and symptoms and the impact of persistently low ALP adjusted for age and sex <sup>2</sup>

\*In adults (n=52) median age of earliest reported manifestations of HPP was 37.6 years (min: 0.2, max: 75.2), which preceded median age at diagnosis (47.5 years; min: 0.2, max: 75.2).<sup>1</sup> Data from the Global HPP Registry, sponsored by Alexion Pharmaceuticals, an observational, longitudinal, multinational, long-term study collecting data on HPP diagnosis, history, clinical course, symptoms (including multisystemic aspects of disease) and burden of illness from patients who have a diagnosis of HPP.<sup>1,3</sup>

ALP, alkaline phosphatase; HPP, hypophosphatasia.

1. Högler W, et al. *BMC Musculoskelet Disord*. 2019;20(1):80. 2. Khan AA, et al. *Osteoporos Int*. 2024;35(3):431–438. 3. Seefried L, et al. *J Bone Miner Res*. 2020;35(11):2171–2178.

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**75%** of adult patients with HPP\* suffer from pain, making it one of the most common symptoms<sup>1</sup>



**Neuropathic pain<sup>2,3</sup>**



**Musculoskeletal pain<sup>1,4</sup>**

Rethink HPP disease burden especially the impact on daily function and QoL, and the potential for progression in children and adults<sup>5-7</sup>

\*(N=160/213) Data from the Global HPP Registry, sponsored by Alexion Pharmaceuticals, an observational, longitudinal, multinational, long-term study collecting data on HPP diagnosis, history, clinical course, symptoms (including multisystemic aspects of disease) and burden of illness from patients who have a diagnosis of HPP.<sup>1,6</sup> HPP, hypophosphatasia; QoL, quality of life.

1. Dahir KM, et al. *Orphanet J Rare Dis.* 2022;17(1):277.
2. Lehane F, et al. *J Clin Med.* 2024;13(8):2263.
3. Colazo JM, et al. *Osteoporos Int.* 2019;30(2):469–480.
4. Khan AA, et al. *Osteoporos Int.* 2024;35(3):431–438.
5. Rush ET, et al. *Orphanet J Rare Dis.* 2019;14(1):201.
6. Seefried L, et al. *J Bone Miner Res.* 2020;35(11):2171–2178.
7. Szabo SM, et al. *Orphanet J Rare Dis.* 2019;14(1):85.

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# MORE THAN HALF OF PATIENTS

with HPP may not have overt bone manifestations\*<sup>1</sup>



Neurological,<sup>†</sup> Pain, Fatigue,<sup>1,2</sup>  
Muscle weakness<sup>1</sup>



+ bone  
manifestations<sup>‡</sup>

Rethink HPP disease burden especially the impact on daily function and QoL, and the potential for progression in children and adults<sup>3-5</sup>

\*Skeletal manifestations were present in 44.2% (N=53/120) of paediatric (<18 years) and 48.2% (N=66/137) of adult (≥18 years) patients with HPP.<sup>1</sup> Data from the Global HPP Registry, sponsored by Alexion Pharmaceuticals, an observational, longitudinal, multinational, long-term study collecting data on HPP diagnosis, history, clinical course, symptoms (including multisystemic aspects of disease) and burden of illness from patients who have a diagnosis of HPP.<sup>1,5</sup> <sup>†</sup>Includes craniosynostosis, developmental delay, increased intracranial pressure and seizures.<sup>1</sup> <sup>‡</sup>Includes bone deformity, pseudofractures, recurrent and poorly healing fractures, rickets-like changes (by radiograph).<sup>1</sup> HPP, hypophosphatasia; QoL, quality of life.

1. Högler W, et al. *BMC Musculoskelet Disord*. 2019;20(1):80.
2. Dahir KM, et al. *Orphanet J Rare Dis*. 2022;17(1):277.
3. Rush ET, et al. *Orphanet J Rare Dis*. 2019;14(1):201.
4. Seefried L, et al. *J Bone Miner Res*. 2020;35(11):2171–2178.
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# Patients with and without skeletal manifestations\* have similar impairments in:<sup>1</sup>



**Disability  
scores<sup>†</sup>**



**QoL<sup>‡</sup>**

## Rethink HPP disease burden especially the impact on daily function and QoL, and the potential for progression in children and adults<sup>2-4</sup>

\*Of 468 adults (skeletal group, n=300; muscular/pain group, n=73) from the Global HPP Registry, sponsored by Alexion Pharmaceuticals, an observational, longitudinal, multinational, long-term study collecting data on HPP diagnosis, history, clinical course, symptoms (including multisystemic aspects of disease) and burden of illness from patients who have a diagnosis of HPP.<sup>1,3</sup> <sup>†</sup>HAQ-DI disability assessment scores at baseline; N=191/239 (skeletal group) and N=47/239 (muscular/pain group).<sup>1</sup> <sup>‡</sup>SF-36v2 domain scores at baseline. The individual SF-36v2 domain scores, except the physical functioning score, did not differ between the skeletal (N=191/238) and muscular/pain (N=47/238) groups.<sup>1</sup> HAQ-DI, health assessment questionnaire–disability index; HPP, hypophosphatasia; QoL, quality of life; SF-36v2, Short Form Health Survey version 2.

**1.** Dahir KM, et al. *Front Endocrinol (Lausanne)*. 2023;14:1138599. **2.** Rush ET, et al. *Orphanet J Rare Dis*. 2019;14(1):201. **3.** Seefried L, et al. *J Bone Miner Res*. 2020;35(11):2171–2178. **4.** Szabo SM, et al. *Orphanet J Rare Dis*. 2019;14(1):85.

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