# TRACKING YOUR HPP

Hypophosphatasia, or HPP, is an inherited metabolic disease that may progress over time and have a lifelong impact.

It is an uncommon disease and can affect patients of any age, from infants to adults.

## WHAT DOES HPP LOOK LIKE?

**People with HPP can experience symptoms in their bones, muscles, joints, teeth, lungs, brain, and kidneys**

**Symptoms of HPP may become worse over time, and new symptoms can appear at any age**

This kit will help you and your doctor develop a more complete picture of your medical history related to HPP by providing a place to record your signs and symptoms at diagnosis and over time



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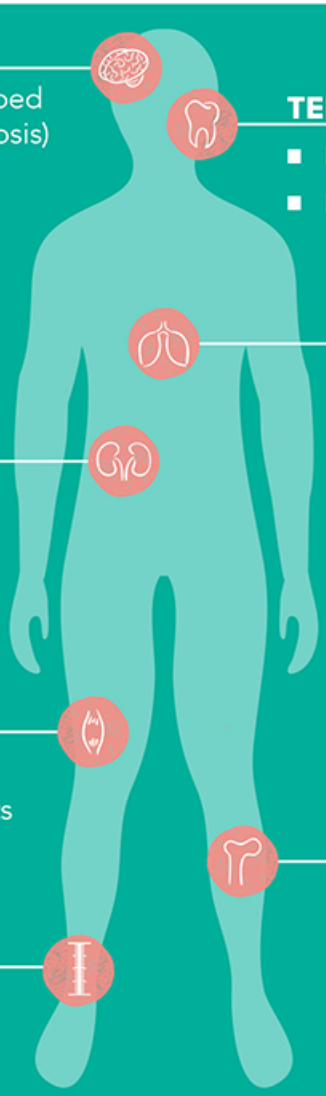
# UNDERSTANDING YOUR HPP SIGNS AND SYMPTOMS IS KEY TO MANAGING THE CONSEQUENCES

## POTENTIAL CONSEQUENCES OF HPP



### SKULL AND BRAIN

- An abnormally shaped head (craniosynostosis)
- Seizures
- Headaches

### TEETH

- Early tooth loss with the root intact
- Poor dental health/cavities

### RIBS AND LUNGS

- An abnormally shaped chest, which can lead to underdeveloped lungs
- Difficulty breathing and the need for breathing support

### KIDNEYS

- A buildup of calcium in the kidneys
- Kidney disease or kidney failure

### MUSCLES AND JOINTS

- Muscle weakness
- Pain in the muscles or joints

### BONES

- Softening of bones (rickets due to HPP/osteomalacia)
- Weak or brittle bones
- Frequent fractures
- Bowed legs/knock-knees
- Bone pain

### GROWTH/DEVELOPMENT

- Delayed growth/weight gain (failure to thrive)
- Short stature
- Delays in developmental milestones[a]:
  - Rolling over
  - Sitting
  - Crawling
  - Standing
  - Walking
  - Climbing stairs

## YOUR DOCTOR MAY ORDER ADDITIONAL TESTS TO MONITOR SIGNS AND SYMPTOMS YOU MAY BE EXPERIENCING

[a]Talk to your doctor about what constitutes a delay for each of these milestones.

This information is intended as educational information for patients and their doctors. It does not replace a doctor's judgment or clinical diagnosis. Speak with your doctor about your medical condition and any specific symptoms that you may be experiencing.



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# TRACKING HPP SYMPTOMS

Tracking your symptoms is an important part of understanding the progression of HPP and provides useful information for your doctor to provide the best care for you. While the tools included will help track your symptoms, your doctor may ask you to complete other assessments and may conduct additional testing.

1. Fill out one of the health questionnaires when you first receive this folder. Label it "Baseline" next to the date and place it in the back pocket to keep for your records.

   - With the help of a parent or legal guardian, children under the age of 18 should fill out the CHAQ form (Childhood Health Assessment Questionnaire)
     - **CHAQ:** Questionnaire developed to assess health status for children with arthritis. Although not specific to HPP, this may be a helpful resource to assess health status in children.
   - Adults 18 years and older, with HPP beginning ≤18 years of age, should fill out the Adult HPP Assessment Questionnaire
     - **Adult HPP Assessment Questionnaire:** Questionnaire to help evaluate patient-reported symptomatology and burden of disease of HPP in adults. This encompasses the 12-item Short Form Health Survey Questionnaire version 2 (SF-12v2) as well as additional adapted questions from the Hypophosphatasia Impact Patient Survey (HIPS) and the Hypophosphatasia Outcomes Study Telephone interview (HOST), which were developed to capture patients' HPP-related medical history and health-related quality of life

2. Fill out a new form before every follow-up appointment, and bring it with you to discuss with your doctor.

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# HPP HISTORY

**DATE** _____

| SYMPTOM HISTORY | | AGE SYMPTOM FIRST APPEARED | | FAMILY HISTORY |
|---|---|---|---|---|
| **SKELETAL (BONE)** | | | | |
| Weak or brittle bones | | | | |
| Bone deformities, including of the skull and chest | | | | |
| Fractures | | | | |
| Bowed legs/knock-knees | | | | |
| Bone pain | | | | |
| Softening of bones (osteomalacia or rickets) | | | | |
| **RESPIRATORY (LUNGS)** | | | | |
| Difficulty breathing (eg, need for breathing support) | | | | |
| **DENTAL (TEETH)** | | | | |
| Early tooth loss with the root intact | | | | |
| Poor dental health/cavities | | | | |
| **MUSCULAR/MOVEMENT** | | | | |
| Muscle weakness | | | | |
| Low muscle tone (hypotonia) | | | | |
| Muscle/joint pain | | | | |
| Difficulty walking (waddling gait) | | | | |
| **BRAIN** | | | | |
| Seizures | | | | |
| **GROWTH/DEVELOPMENT** | | | | |
| Delayed growth/weight gain (failure to thrive) | | | | |
| Delayed/missed developmental milestones[a] | | | | |
| Short stature | | | | |
| **ASSISTANCE** | | | | |
| Trouble walking or need for mobility assistance (circle any used) Wheelchair/walker/crutches/cane | | | | |

[a]Examples include trouble with motor skills, such as sitting, crawling, walking, running, jumping, climbing, etc. Talk to your doctor about what constitutes a delay for developmental milestones.

## PAST LAB RESULTS

If you do not have this information, ask your doctor to help fill in this section.

| | RESULTS | TEST DATES |
|---|---|---|
| ALP (alkaline phosphatase) | | |
| PLP (vitamin B6) | | |

PLP, pyridoxal-5'-phosphate.



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# CHAQ – CHILDHOOD HEALTH ASSESSMENT QUESTIONNAIRE

## (Patients <18 years of age)

Patient name: _____ Date: _____

Person completing: ☐ Mother ☐ Father ☐ Patient ☐ Other: _____

In this section, we are interested in learning how your child's illness affects his/her ability to function in daily life. In the following questions, please check the one response that best describes your child's usual activities (average over an entire day) **OVER THE PAST WEEK.** ONLY NOTE THOSE DIFFICULTIES OR LIMITATIONS THAT ARE DUE TO ILLNESS. If most children at your child's age are not expected to do a certain activity, please mark "Not Applicable." For example, if your child has difficulty doing a certain activity or is unable to do it because he/she is too young but NOT because he/she is RESTRICTED BY ILLNESS, please mark "Not Applicable."

| | Without ANY Difficulty | With SOME Difficulty | With MUCH Difficulty | UNABLE to Do | Not Applicable |
|---|---|---|---|---|---|
| **Dressing and Grooming** | | | | | |
| Is your child able to: | | | | | |
| Dress, including tying shoelaces and doing buttons? | ☐ | ☐ | ☐ | ☐ | ☐ |
| Shampoo his/her hair? | ☐ | ☐ | ☐ | ☐ | ☐ |
| Cut fingernails? | ☐ | ☐ | ☐ | ☐ | ☐ |
| **Arising** | | | | | |
| Is your child able to: | | | | | |
| Stand up from a low chair or floor? | ☐ | ☐ | ☐ | ☐ | ☐ |
| Get in and out of bed or stand up in crib? | ☐ | ☐ | ☐ | ☐ | ☐ |
| **Eating** | | | | | |
| Is your child able to: | | | | | |
| Cut his/her own meat? | ☐ | ☐ | ☐ | ☐ | ☐ |
| Lift a cup or glass to mouth? | ☐ | ☐ | ☐ | ☐ | ☐ |
| Open a new cereal box? | ☐ | ☐ | ☐ | ☐ | ☐ |
| **Walking** | | | | | |
| Is your child able to: | | | | | |
| Walk outdoors on flat ground? | ☐ | ☐ | ☐ | ☐ | ☐ |
| Climb up 5 steps? | ☐ | ☐ | ☐ | ☐ | ☐ |
| **Activities** | | | | | |
| Is your child able to: | | | | | |
| Run errands and shop? | ☐ | ☐ | ☐ | ☐ | ☐ |
| Get in and out of car? | ☐ | ☐ | ☐ | ☐ | ☐ |
| Ride bike or tricycle? | ☐ | ☐ | ☐ | ☐ | ☐ |
| Run and play? | ☐ | ☐ | ☐ | ☐ | ☐ |
| Do household chores (wash dishes, take out trash, vacuum, make bed)? | ☐ | ☐ | ☐ | ☐ | ☐ |

**Please check any AIDS or DEVICES that your child usually uses for any of the above activities**

☐ Cane ☐ Crutches ☐ Built-up pencil/special utensils ☐ Devices used for dressing (button hook, zipper pull, etc)

☐ Walker ☐ Wheelchair ☐ Special/built-up chair ☐ Other: _____



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| | Without ANY Difficulty | With SOME Difficulty | With MUCH Difficulty | UNABLE to Do | Not Applicable |
|---|---|---|---|---|---|
| **Hygiene** | | | | | |
| Is your child able to: | | | | | |
| Wash and dry entire body? | ☐ | ☐ | ☐ | ☐ | ☐ |
| Get in and out of the tub? | ☐ | ☐ | ☐ | ☐ | ☐ |
| Get on and off the toilet/ potty seat? | ☐ | ☐ | ☐ | ☐ | ☐ |
| Brush teeth? | ☐ | ☐ | ☐ | ☐ | ☐ |
| Comb/brush hair? | ☐ | ☐ | ☐ | ☐ | ☐ |
| **Reach** | | | | | |
| Is your child able to: | | | | | |
| Reach and get down a heavy object such as a large game or book from above his/her head? | ☐ | ☐ | ☐ | ☐ | ☐ |
| Bend down to pick up clothing or a piece of paper from the floor? | ☐ | ☐ | ☐ | ☐ | ☐ |
| Pull a sweater over his/her head? | ☐ | ☐ | ☐ | ☐ | ☐ |
| Turn neck to look back over shoulder? | ☐ | ☐ | ☐ | ☐ | ☐ |
| **Grip** | | | | | |
| Is your child able to: | | | | | |
| Write or scribble with a pen or pencil? | ☐ | ☐ | ☐ | ☐ | ☐ |
| Open car doors? | ☐ | ☐ | ☐ | ☐ | ☐ |
| Open jars that have previously been opened? | ☐ | ☐ | ☐ | ☐ | ☐ |
| Turn faucets on and off? | ☐ | ☐ | ☐ | ☐ | ☐ |
| Push open a door when he/she has turned the knob? | ☐ | ☐ | ☐ | ☐ | ☐ |

**Please check any AIDS or DEVICES that your child usually uses for any of the above activities**

☐ Bathtub seat ☐ Raised toilet seat ☐ Long-handled appliances for reach

☐ Bathtub bar ☐ Jar opener (for jars previously opened) ☐ Long-handled appliances for bathroom

**Please check any category for which your child needs help from another person because of illness**

☐ Dressing and grooming ☐ Arising ☐ Eating ☐ Walking

☐ Gripping and opening things ☐ Hygiene ☐ Reach ☐ Errands and chores

**How much pain do you think your child has had because of his or her illness IN THE PAST WEEK?**

No pain — Very severe pain

0 — 100

**Considering all the ways that HPP affects your child, mark how your child is doing on the following scale**

Very well — Very poorly

0 — 100

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# ADULT HPP ASSESSMENT QUESTIONNAIRE

## (Patients ≥18 years of age, with HPP beginning at ≤18 years of age)

This questionnaire asks you about your health. This information will help keep track of how you feel and how well you are able to do your usual activities.

For each of the following questions, please mark an "X" in the one box that best describes your answer.

Patient name: _____ Date: _____

1. In general, would you say your health is:

| Excellent | Very good | Good | Fair | Poor |
|---|---|---|---|---|
| ☐1 | ☐2 | ☐3 | ☐4 | ☐5 |

2. The following questions are about activities you might do during a typical day. Does **your health now limit you** in these activities? If so, how much?

| | Yes, limited a lot | Yes, limited a little | No, not limited at all |
|---|---|---|---|
| a. **Moderate activities** such as moving a table, pushing a vacuum cleaner, bowling, or playing golf | ☐1 | ☐2 | ☐3 |
| b. Climbing **several** flights of stairs | ☐1 | ☐2 | ☐3 |

3. During the **past 4 weeks**, how much of the time have you had any of the following problems with your work or other regular daily activities **as a result of your physical health**?

| | All of the time | Most of the time | Some of the time | A little of the time | None of the time |
|---|---|---|---|---|---|
| a. **Accomplished less** than you would like | ☐1 | ☐2 | ☐3 | ☐4 | ☐5 |
| b. Were limited in the **kind** of work or other activities | ☐1 | ☐2 | ☐3 | ☐4 | ☐5 |

4. During the **past 4 weeks**, how much of the time have you had any of the following problems with your work or other regular daily activities **as a result of any emotional problems** (such as feeling depressed or anxious)?

| | All of the time | Most of the time | Some of the time | A little of the time | None of the time |
|---|---|---|---|---|---|
| a. **Accomplished less** than you would like | ☐1 | ☐2 | ☐3 | ☐4 | ☐5 |
| b. Did work or activities **less carefully than usual** | ☐1 | ☐2 | ☐3 | ☐4 | ☐5 |

5. During the **past 4 weeks**, how much did **pain** interfere with your normal work (including work outside the home and housework)?

| Not at all | A little bit | Moderately | Quite a bit | Extremely |
|---|---|---|---|---|
| ☐1 | ☐2 | ☐3 | ☐4 | ☐5 |

6. These questions are about how you feel and how things have been with you **during the past 4 weeks**. For each question, please give the one answer that comes closest to the way you have been feeling. How much of the time during the **past 4 weeks...**

| | All of the time | Most of the time | Some of the time | A little of the time | None of the time |
|---|---|---|---|---|---|
| a. Have you felt calm and peaceful? | ☐1 | ☐2 | ☐3 | ☐4 | ☐5 |
| b. Did you have a lot of energy? | ☐1 | ☐2 | ☐3 | ☐4 | ☐5 |
| c. Have you felt down-hearted and depressed? | ☐1 | ☐2 | ☐3 | ☐4 | ☐5 |

7. During the **past 4 weeks**, how much of the time has your **physical health or emotional problems** interfered with your social activities (like visiting friends, relatives, etc.)?

| All of the time | Most of the time | Some of the time | A little of the time | None of the time |
|---|---|---|---|---|
| ☐1 | ☐2 | ☐3 | ☐4 | ☐5 |

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# ADULT HPP ASSESSMENT QUESTIONNAIRE

## (Patients ≥18 years of age, with HPP beginning at ≤18 years of age)

Patients with HPP and their caregivers may not fully realize all the ways in which HPP is affecting their physical function and quality of life. They may also be unaware that certain signs or symptoms could actually be caused by HPP. The following questions may help highlight limitations and disease impact you may be experiencing.

Patient name: _____ Date: _____

Which 3 symptoms currently bother you the most?

1. _____
2. _____
3. _____

Do you have pain? Yes __ No__

Do you need to use pain medication? Yes __ No__

Have your symptoms impacted your ability to walk and get around? Yes __ No__

- Has this worsened over time? Yes __ No__
- Have you been dependent on any assistive device, such as a cane, crutches, a walker, or a wheelchair, to get around? Yes __ No__

Have your symptoms impacted your ability to perform other activities of daily living such as the following?

- Standing from a sitting position Yes __ No__
- Climbing stairs Yes __ No__
- Descending stairs Yes __ No__
- Picking up objects Yes __ No__
- Reaching above your head Yes __ No__

Have you ever fractured a bone? Yes __ No__

- At what age did you first experience a fracture? _____
- How many fractures have you had? _____
- When was your most recent fracture? _____

Have you been hospitalized or required surgery because of your condition?

- Surgery Yes __ No__
- Hospitalized Yes __ No__

Have you had to miss work or miss participating in certain types of family/social activities because of your condition?

Yes __ No__

Are there any activities that you previously enjoyed but can no longer participate in due to reduced mobility, speed, or agility, and/or excessive pain/fatigue afterward?

Yes __ No__



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