HYPOPARATHYROIDISM

- A rare endocrine disorder caused by absent or insufficient parathyroid hormone (PTH), a critical regulator of serum calcium, phosphate, and active vitamin D levels
- An estimate of the overall prevalence of hypoparathyroidism in Europe is not available, however the prevalence in Denmark has recently been estimated at 25–100 individuals
- Patients with hypoparathyroidism suffer from symptoms of hypocalcemia (eg, numbness, paresthesias, tetany, cognitive difficulties, seizures, cardiac arrhythmias, and laryngospasm/bronchospasm) and complications such as skeletal abnormalities, renal calcifications potentially leading to renal failure, and soft tissue calcifications
- Most commonly caused by injury to the parathyroid gland during surgery
- Current management aims for symptom control with calcium and calcitriol/calciocodiol supplementation
- No PTH replacement treatment has been approved to treat hypoparathyroidism

DATA COLLECTION (cont’d)

- Patient data collected from routine medical care will be recorded in electronic case report forms (eCRFs) using an electronic data capture system
- To ensure quality control and source data verification of eCRFs, 10% of study centers will be visited by a monitor; additional data monitoring may be conducted based on periodic data review
- Although no predetermned patient follow-up requirements are specified by the registry protocol, investigators are expected to update patient data in the registry at each patient visit and at a minimum of every 12 months

OBJECTIVE OF THE REGISTRY

- To characterize the natural history and epidemiology of chronic hypoparathyroidism in patients under conditions of normal clinical practice, regardless of disease origin and management
- Aspects of hypoparathyroidism to be examined include:
  - Etiology
  - Treatment patterns
  - Clinical course and outcomes
  - Comorbidity
  - Mortality
  - Patients’ quality of life (SF-36 Survey)
  - Impact of hypoparathyroidism on patients’ work productivity
  - Hospital, emergency room, and physician visits

RATIONAL FOR THE REGISTRY

- PARADIGHM™: Physicians Advancing Disease Knowledge in Hypoparathyroidism
- Limited published data are available regarding the natural history and epidemiology of chronic hypoparathyroidism,
  - Descriptive epidemiology
  - Risk factors
  - Modifying factors
  - Comorbidities and long-term consequences including nephrocalcinosis, renal failure, and mortality
  - Management strategies
- Results from the registry are intended to assist healthcare providers in optimizing their clinical decision making through enhanced understanding of the variability, progression, and natural history of chronic hypoparathyroidism

PATIENT ELIGIBILITY

Inclusion Criteria

- Patients with a diagnosis of chronic hypoparathyroidism are eligible for inclusion into the registry, regardless of hypoparathyroidism etiology and management
- Diagnosed with chronic hypoparathyroidism (≥6 months)
- Signed informed consent and medical records release

Exclusion Criteria

- Transient hypoparathyroidism within 6 months of enrollment
- Diagnosed with hypoparathyroidism ≤6 months ago

PARADIGHM™ STUDY DESIGN

- Patients will be enrolled at participating study centers
  - Approximately 7 years of patient recruitment
  - Global target enrollment is 900 patients
  - Estimated study completion date: June 2032
  - ClinicalTrials.gov identifier: NCT01922440
  - Follow-up for each patient will be a minimum of 10 years
  - Patient care and follow-up will be according to routine care by the patients’ physicians
- Except for patient questionnaires, no study-defined procedures will be required

DATA COLLECTION

Table 1. Registry Data to be Collected

<table>
<thead>
<tr>
<th>Data Field</th>
<th>Baseline</th>
<th>Follow-Up</th>
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<tbody>
<tr>
<td>Informed consent and medical records release</td>
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<td>X</td>
</tr>
<tr>
<td>Inclusion/exclusion criteria</td>
<td>X</td>
<td></td>
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<tr>
<td>Demographic information</td>
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<td>Family history of hypoparathyroid</td>
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<td>Hypoparathyroid history</td>
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<td>Medical history</td>
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<td>Prior and concomitant medications (including over-the-counter medications)</td>
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<td>Hypoparathyroid management</td>
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<tr>
<td>Height and weight</td>
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<tr>
<td>Laboratory and imaging evaluations</td>
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<tr>
<td>Patient- or investigator-reported questionnaires (eg, quality of life, social history, hospitalizations)</td>
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<tr>
<td>New concomitant diseases</td>
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</tr>
<tr>
<td>Mortality and cause of death</td>
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</tr>
</tbody>
</table>

REFERENCES


DISCLOSURES

SBJ, TS, and HJ are employees of NPS Pharmaceuticals, Inc. DJ and MM have served as advisory group members for NPS Pharmaceuticals, Inc. JBP, BLC, and DMS have received institutional research grants from and served as advisory group members for NPS Pharmaceuticals, Inc. The PARADIGHM™ registry is supported by NPS Pharmaceuticals, Inc., Bedford, NJ, USA.

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