



# The Nexus of Patients and Science™



JENNY LIVES WITH SHORT BOWEL SYNDROME





At NPS Pharma, patients are at the center of everything we do. Each of us brings a personal passion to fulfilling our mission of pioneering and delivering innovative therapies that transform the lives of patients with rare diseases worldwide.

Our vision is to create a world where every person living with a rare disease has a therapy. We aim to accomplish this in partnership with patients, physicians, researchers, universities, policy makers and companies throughout the globe.

**Together we can make a difference.**

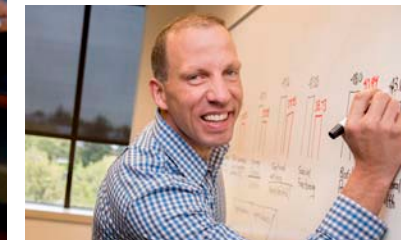
*Pladeau*



**LUCIA** | SENIOR ADMINISTRATIVE ASSISTANT  
*"Colleagues welcome my input and respect my opinions"*



**KAREN** | CARE COORDINATOR (TOP)  
*"I have the privilege of helping the most important stakeholders we have – patients."*



**CHRIS** | EXECUTIVE DIRECTOR (BOTTOM)  
*"I am empowered to make decisions that will shape the future of our global company."*



**ERIC** | DIRECTOR  
*"My work provides measurable value to patients and society."*

Our **values** and **culture** are the most important drivers of our success and will support us in achieving our goal of developing innovative therapies for 10 discrete rare diseases by 2023. Integrity, respect, excellence, personal ownership, teamwork, entrepreneurial spirit and fun are the values that guide our work each day and what we expect from each other.

**They are the fabric of the NPS Pharma culture.**

# About NPS Pharma

NPS Pharma is a global biopharmaceutical company pioneering and delivering innovative therapies that transform the lives of patients with rare diseases. Our vision is a world where every person living with a rare disease has a therapy.

Our current therapeutic areas of focus are rare gastrointestinal disease and endocrine disorders. These include Short Bowel Syndrome, a potentially fatal gastrointestinal disorder in which patients are unable to absorb enough nutrients and fluids through the intestine from the foods they eat to survive; Hypoparathyroidism, a complex endocrine disorder in which the parathyroid glands are either absent or damaged, and the body produces insufficient or no parathyroid hormone; and Autosomal Dominant Hypocalcemia, an ultra-rare, genetic disorder of calcium homeostasis caused by mutations of the calcium-sensing receptor gene.

Our business model is focused on global development and commercialization of orphan products. With worldwide rights to our three rare disease products and an established global infrastructure, we are developing and commercializing these products independently. By design, we do not have discovery capabilities and actively seek in-licensing opportunities to develop new therapies for a broad range of rare diseases. We currently have several royalty-based agreements in place with Amgen, GlaxoSmithKline, Janssen Pharmaceuticals and Kyowa Hakko Kirin for our non-orphan products and product candidates.

For more information visit [npsp.com](https://npsp.com).



We have nearly **400 employees** and operations in the U.S., Canada, Europe, Latin America and Japan. Our global headquarters are located in the center of the world’s biopharmaceutical industry in Bedminster, New Jersey, USA, and our international headquarters are based in Dublin, Ireland.



# Our History

At **NPS Pharma**, we have a rich history spanning nearly three decades that **fuels our vision of a world where every person living with a rare disease has a therapy.**

1986

NPS Pharma founded by Drs. Hunter Jackson and Thomas Parks, two dedicated research scientists from the University of Utah School of Medicine studying the medicinal effects of spider venom.

1993-96

Strategic collaborations with Kyowa Hakko Kirin (previously Kririn Pharma) and Amgen for hyperparathyroidism treatment and GlaxoSmithKline for calcilytic compounds in osteoporosis.

1999

Acquired Allelix Pharmaceuticals, including assets key to the company's current success—investigational treatments for Short Bowel Syndrome and Hypoparathyroidism.

2003-04

Moved headquarters to New Jersey, USA and first NPS Pharma molecule approved for hyperparathyroidism and marketed by Amgen.

2006

Osteoporosis treatment approved in Europe (marketed by Nycomed) is not approved in the U.S., leading to significant company restructuring, reorganizing and refocusing.

2007-08

Under the leadership of our current President and CEO, Dr. Francois Nader, NPS Pharma re-invents itself as a rare disease company with a new business model.

2013-14

NPS Pharma's first product approved and commercialized in the U.S. and Europe for adult Short Bowel Syndrome.

2015

NPS Pharma's second product approved and commercialized in the U.S for Hypoparathyroidism and under review in Europe.



## Rare Disease

People living with rare diseases have incredible courage. They have to, because they have few, if any, treatment options and are often over looked because of the rarity of their disorders. There are approximately 7,000 rare diseases and treatments exist for less than 500 of them. We can do better than this. We must do better than this.

Many rare diseases are severe with life-threatening consequences. They have a profound impact on patients, families and communities, often making it challenging for people to work, care for their families and live independently.

NPS Pharma is the first company to:

- Deliver a prescription medication for the long-term treatment of adults living with Short Bowel Syndrome
- Develop a global pediatric program for Short Bowel Syndrome
- Develop a replacement therapy to treat Hypoparathyroidism
- Initiate a clinical program for Autosomal Dominant Hypocalcemia

There will be many more firsts in our future as we strive to bring hope to people living with the almost 6,500 rare diseases that today are without a treatment.

We are working hard because patients are waiting.



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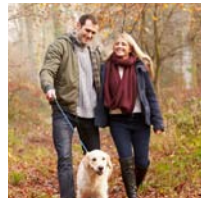


# SBS: Short Bowel Syndrome

Short Bowel Syndrome (SBS) is a potentially fatal rare gastrointestinal disorder where patients are unable to absorb enough nutrients and fluids through the intestine from the foods they eat, leaving many to rely on parenteral nutrition for their survival.

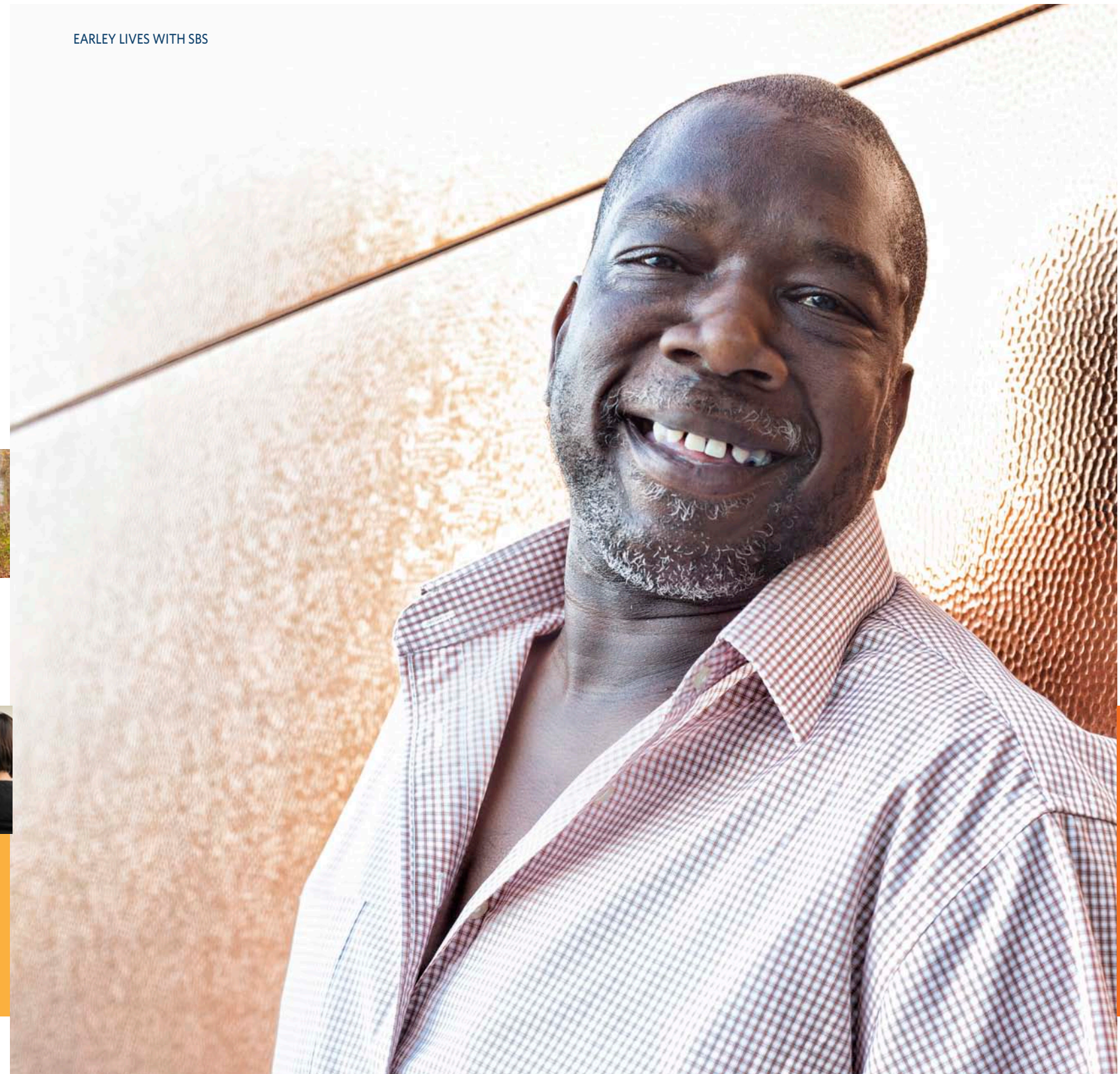
We began studying this rare disorder because we saw a clear opportunity to make a difference for people living with SBS. NPS Pharma has delivered the first long-term treatment advance for SBS in approximately 40 years, and we are currently exploring a pediatric treatment option.

For more information on SBS, please visit [ShortBowelSupport.com](https://ShortBowelSupport.com), a website we created with the help of short bowel patients and their healthcare practitioners to provide useful information about the disease, its symptoms and its management.



NPS Pharma has developed the first prescription medication for the long-term treatment of adults living with SBS, and we are currently exploring a pediatric treatment option.

EARLEY LIVES WITH SBS







## Hypoparathyroidism

Hypoparathyroidism is a rare and complex endocrine disorder in which the parathyroid glands are either absent, or damaged and the body produces insufficient or no parathyroid hormone (PTH). PTH plays a central role in a variety of critical physiological functions, including closely modulating serum calcium and phosphate, regulating renal excretion of calcium and phosphate, activating vitamin D, and maintaining normal bone turnover.

In patients with Hypoparathyroidism, insufficient levels of PTH lead to low serum calcium, high serum phosphate, increased urinary calcium excretion, and decreased urinary phosphorus excretion. PTH deficiency can also disrupt skeletal homeostasis, leading to bone abnormalities. In addition, patients with insufficient levels of PTH are unable to convert native vitamin D into its active state to properly absorb dietary calcium.

NPS Pharma has developed the first parathyroid hormone therapy for people living with Hypoparathyroidism.

For more information about the disorder, please visit [Hypoparathyroidism.com](https://hypoparathyroidism.com), a website we created with the help of Hypoparathyroidism patients and their healthcare practitioners to provide useful information about the disorder, its symptoms and its management.



NPS Pharma has developed the first parathyroid hormone therapy for people living with Hypoparathyroidism.



# ADH: Autosomal Dominant Hypocalcemia

Autosomal Dominant Hypocalcemia, or ADH, is an ultra-rare, genetic disease caused by mutations of the calcium-sensing receptor (CaSR) gene that increases the sensitivity of the receptor to serum calcium.

The underlying cause of symptoms and complications in patients are two-fold. First, absolute hypocalcemia can cause life-threatening seizures, laryngeal spasms, cardiac arrhythmias, tetany, paresthesias, muscle cramping, and decreased focus. In addition, the body's perceived hypercalcemia results in inappropriately increased renal calcium excretion, decreased renal phosphate excretion, and hyperphosphatemia, putting patients at risk for renal complications, including renal stones, nephrocalcinosis, and impaired renal function, as well as soft tissue calcifications. Currently, there are no approved treatment options for patients with ADH.

NPS Pharma is conducting a Phase 2a study evaluating a potential treatment for people living with ADH.

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## Partnering to Address Unmet Needs

**As a patient-centric, values-driven company focused exclusively on rare disease, we are building a global pipeline of first-in- or best-in-disease therapies for patients with significant unmet medical needs. Our vision of creating a world where every person living with a rare disease has a therapy drives our long-term goal of developing innovative treatments to patients suffering from 10 distinct rare diseases in 10 years.**

**With worldwide rights to our three rare disease products and an established global infrastructure, we are developing and commercializing these products independently. By design we do not have discovery capabilities and partnership is a key pillar of our growth.**

**We are seeking partners with innovative therapies, including both large and small molecules, that will transform the lives of people with rare diseases.**

**We currently have several successful partnerships in place with Amgen, GlaxoSmithKline, Janssen Pharmaceuticals, and Kyowa Hakko Kirin for our non-orphan products and product candidates.**

To learn more, please visit: [nps.com/partners](http://nps.com/partners).

**Treatments for** **10** **Diseases**  
**in**  
**10** **Years**



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\*Please see website for additional offices

