
EXECUTIVE SUMMARY

Management Team:

Rachael Hagan, M.Sc.
President & CEO, Chairman

Recognized leader with demonstrated track record of commercializing early-stage technology out of universities.

Jeff M. Sands, M.D.
Founder, Director

Director, Emory Renal Division, Professor of Medicine and Physiology, President Elect, American Physiological Society, Key Opinion Leader for NDI.

Janet D. Klein, Ph.D.
Founder

Associate Professor of Medicine and Physiology, Renal Division, Emory, Recognized leading expert on the cellular and molecular mechanisms that contribute to changes in water and solute homeostasis that occur in systemic and kidney diseases.

Ish Khanna, Ph.D.
Founder, Director

Over 28 years of pharmaceutical industry experience, Co-inventor of celecoxib and multiple clinical agents across therapeutics.

Ram Pillarisetti, Ph.D.
Founder

Over 23 years of experience in research in Academia and Industry.

Donna See,
Director

CBO, TARA Biosystems, Inc. Former Vice President at Allied Minds, 14 years of experience in academic technology transfer, public-private partnerships, and early-stage technology development.



Overview: NephroDI Therapeutics, Inc. is an early-stage pharmaceutical company that focuses on concentration disorders of the kidney. The initial clinical indication for its orally administered lead small molecule is for Nephrogenic Diabetes Insipidus in children, a pediatric orphan indication.

Need: Nephrogenic Diabetes Insipidus (NDI) is a disease where patients produce extremely large amounts of dilute urine resulting from an inability of the kidney to respond to vasopressin. Congenital NDI in the pediatric population results primarily from mutations in the type 2 vasopressin receptor (stimulating mechanisms that concentrate the urine and maintain water homeostasis in the organism), which is located on the X chromosome. Congenital NDI has a profound impact on children. Since these children can produce up to 20 Liters of urine per day, they must drink 20 L of water per day to avoid dehydration. Children who suffer multiple episodes of severe dehydration often end up with mental retardation, which can be prevented with adequate water intake.

NDI should not be confused with diabetes mellitus (i.e. sugar diabetes). Despite the similar name, the two conditions are completely different. Diabetes is an abnormal state marked by passage of excessive amounts of urine. NDI does not involve disorders of blood-glucose control.

Congenital NDI manifests at birth and is a life-long condition with a normal life expectancy. It is considered an orphan condition in the US and Europe: the best estimate is that for every 1 million males born in the US, four are likely to have X-linked congenital NDI.

Currently there is no effective therapy for NDI. Current management requires patients to drink as much fluid as they excrete to prevent dehydration. Patients are also prescribed a thiazide diuretic and must maintain an extremely low sodium diet (0.5 g/day). While often onerous, especially the ultra-low-sodium diet, these measures are minimally effective. They may also be prescribed non-steroidal anti-inflammatory drugs or NSAIDs, but these can cause additional kidney damage with chronic use. To maintain a semblance of a normal lifestyle, NDI patients can succumb to the temptation to void less frequently. This behavior can result in an enlarged bladder, which can cause an obstructive nephropathy and lead to kidney failure. There is a compelling clinical need for an effective therapy. A therapy that significantly reduces urine output would be a tremendous benefit to children suffering from NDI.

Product: Our lead product, NDI-5033 is a unique adenosine monophosphate activated kinase (AMPK) activator that can stimulate water reabsorption without causing hypoglycemia. NDI-5033 is currently entering preclinical development.

