



Metabolic Disorders

The endocrine system is an electrochemical connection between the hypothalamus of the brain and the organs of the body that control metabolism, growth and development, and reproduction. It is made up of organs called glands, which secrete chemicals called hormones. Hormones are messengers that travel throughout the body via the bloodstream, affecting other processes and pathways. Typical glands of the endocrine system include the pituitary, thyroid, and adrenal glands. The metabolic pathway is influenced by the endocrine system. It involves the building and breaking down many of the molecules that the body consumes and produces. The endocrine system is involved in nearly every internal process, from digestion to growth to the balance of fluids and nutrients in the body.

Acromegaly

This condition is due to the production of too much growth hormone by the pituitary gland after the end of adolescence, resulting in gigantism. People with pituitary gigantism can truly be giants; they can sometimes end up over 7 or 8 feet in height. The manifestations of acromegaly include thickening of the skin, soft tissues, and bones of the hands and feet. These effects are insidious and very slowly progressive. Ultimately they cause considerable disability including hoarseness, sleep apnea, joint pain, cardiovascular disease, hypertension, insulin resistance, visual impairment and severe headaches.

Indication	Company	Product	Phase
Acromegaly	Tercica Inc	Somatuline Autogel (lanreotide)	PA

Addison’s Disease

Long-term under functioning of the outer portion of the adrenal gland, Addison’s disease is chronic insufficiency of the adrenal cortex. Causes may be due to a number of different insults to the adrenal including physical trauma, hemorrhage, and tuberculosis of the adrenal, and destruction of the cells in the pituitary gland that secrete ACTH (adrenocorticotrophic hormone) which normally drives the adrenal. Addison’s disease is characterized by bronzing of the skin, anemia, weakness, and low blood pressure.

Indication	Company	Product	Phase
Addison’s Disease	Impax Laboratories Inc	Fludrocortisone Acetate	M

Appetite

In order for the body to function, it needs calories and nutrients obtained from food. For many, this is not a problem. The body gives signals such as hunger pangs to let one know that is time to eat. An appetite is regulated closely by the brain, digestive tract, and adipose (fat) tissue. Many disorders, both biological and psychological, exist in which appetite regulation is abnormal. This can lead to diseases such as anorexia and bulimia where too little food intake occurs and obesity where too much food is consumed.

Indication	Company	Product	Phase
Appetite	Pherin Pharmaceuticals Inc	PH284	II

Diabetes

Normally, blood glucose levels are tightly controlled by insulin, a hormone produced by the pancreas. Insulin lowers the blood glucose level. When the blood glucose elevates (for example, after eating food), insulin is released from the pancreas to normalize the glucose level. In patients with diabetes, the absence or insufficient production of insulin causes hyperglycemia. Diabetes is a chronic medical condition, meaning that although it can be controlled, it lasts a lifetime. Over time, diabetes can lead to blindness, kidney failure, and nerve damage, resulting from damage to small vessels.

Company	Product	Phase	Indication	
Impax Laboratories Inc	Metformin HCl Extended Release Tablets	A	Type II Diabetes	
Oculus Innovative Sciences Inc	Dermacyn	II	Diabetic Foot Infection	
Genentech Inc	Topical VEGF / PRO128115	II	Diabetic Foot Ulcers	
Genentech Inc	Lucentis (ranibizumab)	II	Diabetic Macular Edema	
Depomed Inc	Gabapentin ER / Gabapentin GR	II	Diabetic Neuropathy	
NeurogesX Inc	Transacin (capsaicin)	II		
Sangamo BioSciences Inc	SB-509	II		
CoMentis Inc	ATG002	II		
Genentech Inc	Topical VEGF / PRO128115	II		
Bayhill Therapeutics Inc	BHT-3021	II	Type I Diabetes	
BEXEL Pharmaceuticals Inc	BLX-1002	II	Type II Diabetes	
DiObex Inc	DIO-902	II		
InteKrin Therapeutics Inc	AMG131 / INT131	II		
Metabolex Inc	MBX-2044	II		
Napo Pharmaceuticals Inc	NP-500	II		
Perlegen Sciences Inc	MCC-555 (netoglitzazone)	II		
Plexxikon Inc	PLX204	II		
SuperGen Inc	PZG (pyrazinoylguanidine)	II		
Theracos Inc	THR-0921	II		
Aradigm Corp	AERx insulin Diabetes Management System	III		Type I Diabetes
Aradigm Corp	AERx insulin Diabetes Management System	III		Type II Diabetes
Metabolex Inc	MBX-102 / JNJ-39659100 (metaglidase)	III		Type II Diabetes
Nektar Therapeutics	Exubera (inhaled insulin)	M	Diabetes	
Depomed Inc	Glumetza / Metformin GR (metformin hydrochloride)	M	Type II Diabetes	

Growth Hormone Deficiency

Growth Hormone is manufactured in the pituitary gland. If missing or reduced in quantity during infancy or childhood, it results in growth retardation, short stature and other maturation delays. Growth Hormone Deficiency (GHD) causes an absence or delay of the lengthening and widening of the skeletal bones appropriate to the chronological age of the child. A sufficient quantity of growth hormone is required during childhood to maintain growth and normalize sexual maturity.

Indication	Company	Product	Phase
Growth Hormone Deficiency	Genentech Inc	ALTU-238 (somatropin)	II
	Tercica Inc	Increlex (mecasermin [rDNA origin] injection) / rhIGF-1	III
	Genentech Inc	Nutropin [somatropin (rDNA origin) for injection]	M
	Genentech Inc	Nutropin AQ [somatropin (rDNA origin) injection]	M
	Tercica Inc	Nutropin AQ [somatropin (rDNA origin) injection]	M

Homocystinuria

Homocystinuria is a rare metabolic condition characterized by an excess of the compound homocystine in the urine. The condition may result from deficiency of any of several enzymes involved in the conversion of the essential amino acid methionine to another amino acid cysteine, or less commonly, the impaired conversion of the compound homocysteine to methionine. Enzymes are proteins that accelerate the rate of chemical reactions in the body. Certain amino acids, which are the chemical building blocks of proteins, are essential for proper growth and development. Due to deficiency of the CBS enzyme, infants with Homocystinuria may fail to grow and gain weight at the expected rate.

Indication	Company	Product	Phase
Homocystinuria	Jazz Pharmaceuticals	Cystadane (betaine anhydrous)	M

Hypoglycemia

Hypoglycemia is produced by low blood sugar. It occurs when the level of sugar, or glucose, in the blood drops too low to fuel the body. Hypoglycemia is not a disease but a condition that results from a variety of causes. Fasting low blood sugar (hypoglycemia) occurs when you have not eaten for many hours, such as overnight. It is frequently associated with having too much insulin in the blood, some medications, alcohol use, hereditary problems with the metabolizing of carbohydrates, and other health conditions. Nonfasting low blood sugar (also called reactive or postprandial hypoglycemia) develops within three to four hours after eating. It can be caused by hereditary enzyme deficiencies such as galactosemia or by surgery to remove all or part of the stomach.

Company	Product	Phase	Indication
DiObex Inc	DIO-901 (VLD-glucagon)	II	Hypoglycemia

Idiopathic Short Stature

Idiopathic Short Stature (ISS) refers to the condition of someone being shorter in height than those around them. Height, however, is a relative condition, and there are no established definitions as to what is a “normal” height. There have been several controversies over this issue regarding quality of life due to height and insurance reimbursement for hormone therapies and treatments.

Company	Product	Phase	Indication
Genentech Inc	Nutropin [somatropin (rDNA origin) injection]	M	Idiopathic Short Stature
Genentech Inc	Nutropin AQ [somatropin (rDNA origin) injection]	M	

Testosterone Replacement Therapy

Testosterone is a steroid hormone primarily secreted in the testes of males and the ovaries of females. The adrenal glands also secrete small amounts of the hormone. It is the principal male sex hormone and an anabolic steroid which plays major roles in health and well-being. Libido, energy levels, immune function, protection against osteoporosis, and puberty in males are largely due to increased levels of testosterone in the body. In some individuals, however, the production of testosterone is insufficient for the healthy growth and development of the body. In these cases, hormone replacement therapy is used to maintain normal levels of testosterone in the body.

Company	Product	Phase	Indication
Impax Laboratories Inc	Methitest CIII (methyltestosterone)	M	Testosterone Replacement Therapy
Impax Laboratories Inc	Testoderm TTS (testosterone)	M	