

- 2005**
- T-cell acute lymphoblastic leukemia and T-cell lymphoblastic lymphoma: nelarabine
  - chronic iron overload: deferasirox
  - advanced renal cell carcinoma: sorafenib tosylate
  - myelodysplastic disorder: lenalidomide
  - mucopolysaccharidosis VI: galsulfase
  - severe primary IGF-1 deficiency: mecasermin
  - glioblastoma: temozolomide

- secondary hyperparathyroidism: cinacalcet
- malignant pleural mesothelioma: pemetrexed
- acute lymphoblastic leukemia: clofarabine
- pulmonary arterial hypertension: iloprost

**2004**

- 2003**
- CD20-positive follicular non-Hodgkin's lymphoma: tositumomab and Iodine-131 tositumomab
  - Fabry disease: agalsidase beta
  - type I Gaucher disease: miglustat
  - acromegaly: pegvisomant

- hereditary tyrosinemia type I: nitisinone
- Cryptosporidium parvum or Giardia lamblia: nitazoxanide
- cataplexy associated with narcolepsy: sodium oxybate
- pulmonary arterial hypertension: treprostinil

**2002**

- 2001**
- chronic myeloid leukemia: imatinib mesylate
  - pulmonary arterial hypertension: bosentan

- acute promyelocytic leukemia: arsenic trioxide
- acute myeloid leukemia: gemtuzumab

**2000**

- 1999**
- hypoxic respiratory failure in newborns: nitric oxide
  - respiratory distress syndrome in premature infants: poractant alfa
  - anaplastic astrocytoma: temozolomide

- Crohn's disease: infliximab
- pulmonary tuberculosis: rifapentine
- narcolepsy: modafinil
- erythema nodosum leprosum: thalidomide
- thrombocytopenia: lepirudin

**1998**

- 1997**
- pediatric respiratory distress syndrome: calfactant
  - hemophilia B: recombinant human Factor IX

- interstitial cystitis: pentosan polysulfate sodium
- neurocystercosis and hydatid disease: albendazole
- symptomatic orthostatic hypotension: midodrine hydrochloride

**1996**

- 1995**
- amyotrophic lateral sclerosis: riluzole
  - primary pulmonary hypertension: epoprostenol