



2013 Breakthrough Therapies Designation Year-End Update

Designations Granted by the FDA: 37

Requests Denied by the FDA: 70

Designations Announced Publicly by Companies: 28

- Cancer: 12
- Hepatitis C: 4
- Cystic Fibrosis: 2
- Autoimmune Disorders: 2
(Lambert Eaton Myasthenic Syndrome)
(Sporadic inclusion body myositis)
- Heart Disease: 1
- Malaria: 1
- Antidote for Factor XA Inhibitors: 1
- Molybdenum cofactor deficiency: 1
- Duchenne muscular dystrophy: 1
- Hypophosphatasia: 1
- Lysosomal acid lipase deficiency: 1
- Epidermolysis bullosa: 1

Approvals: 3

- Gazyva (obinutuzumab) - (Genetech/Roche) – Approved 11/1/2013 for chronic lymphocytic leukemia (CLL)
- Ibrutinib - (J&J/Pharmacyclics) – Approved 11/13/13 for mantle cell lymphoma
- Sofosbuvir/ledipasvir combination - (Gilead) – Approved 12/6/13 for Hepatitis C