

A DECADE OF Innovation

New Medicines in Development for Rare Diseases



While we have clearly made much progress over the last decade with over 160 new treatments approved for rare diseases, researchers continue to work diligently to find new treatments for patients. Some of these medicines will provide another treatment option for a disease that has few options; others will be the first of their kind to treat a particular rare disease. All provide hope for patients that one day soon a new treatment will be available for them.

Today, many drugs are in development for rare diseases. Below are the numbers of medicines in clinical trials or FDA review for selected rare diseases.

Selected Rare Diseases	Drugs in Development in U.S.
acromegaly	2
acute lymphoblastic leukemia	12
acute myeloid leukemia	34
acute promyelocytic leukemia	2
amyotrophic lateral sclerosis	7
anaplastic astrocytoma	2
chronic myeloid leukemia	22
Crohn's disease	28
Fabry disease	1
glioblastoma	22
interstitial cystitis	4
malignant pleural mesothelioma	8
narcolepsy	1
pulmonary arterial hypertension	6
respiratory distress syndrome	6
secondary hyperparathyroidism	2

