

## **Novel new orphan drugs approved by FDA in 2015**

Adapted from: *Novel New Drugs Summary 2015*, Food and Drug Administration, USA

<http://www.fda.gov/Drugs/DevelopmentApprovalProcess/DrugInnovation/ucm474696.htm>

Regroupement québécois des maladies orphelines, janvier 2016

Drug	Disease	Authorized Canada	INESSS	CADTH
Alecensa	lysosomal acid lipase deficiency			
Cholbam	bile acid synthesis disorders due to single enzyme defects, and for patients with peroxisomal disorders (including Zellweger spectrum disorders)			
Cotellic	melanoma	√		Under evaluation
Cresemba	zygomycosis; invasive aspergillosis			
Darzalex	multiple myeloma			
Empliciti	multiple myeloma			
Farydak	multiple myeloma			
Kanuma	lysosomal acid lipase deficiency			
Lenvima (EISAI)	follicular, medullary, anaplastic, and metastatic or locally advanced papillary thyroid cancer	√		
Natpara	hypoparathyroidism			
Ninlaro	multiple myeloma			
Orkambi (Vertex)	cystic fibrosis	√		Under evaluation
Portrazza	advanced squamous non-small cell lung cancer			
Praxbind	first reversal agent for the anticoagulant Pradaxa			
Repatha (Amgen)	homozygous familial hypercholesterolemia	√	Under evaluation for June 2016	Under evaluation for Jan. 2016
Strensiq (Alexion)	hypophosphatasia	√	Under evaluation for June 2016	Under evaluation
Tagrisso	EGFR T790M mutation-positive metastatic non-small cell lung cancer			
Unituxin	Neuroblastoma			
Upravi	Pulmonary arterial hypertension			
Xuriden	hereditary orotic aciduria			
Yondelis (Janssen)	treatment of patients with ovarian cancer and soft tissue sarcoma	√		Under evaluation