Novel therapeutic drug		Year of approval	Initially approved indication(s) - abbreviated[a]	
INN	**Brand name**		**EU**	**US**
Aclidinium bromide	EU: Eklira Genuair US: Tudorza Pressair	EU: 2012 US: 2012	Maintenance bronchodilator treatment to relieve symptoms in adult patients with COPD	*Maintenance treatment of bronchospasm associated with COPD*
Cangrelor	EU: Kengrexal US: Kengreal	EU: 2015 US: 2015	Reduction of thrombotic CV events in patients with coronary artery disease undergoing percutaneous coronary intervention	For reducing the risk of periprocedural MI, repeat coronary revascularization, and stent thrombosis
Ceftolozane/ tazobactam	Zerbaxa	EU: 2015 US: 2014	**1)** cIAI **2)** cUTI incl. acute pyelonephritis	***1)*** *cIAI used in combination with metronidazole* ***2)*** *cUTI incl. pyelonephritis*
Cobicistat	Tybost	EU: 2013 US: 2014	Pharmacokinetic enhancement of atazanavir or darunavir as part of antiretroviral combination therapy in HIV-1 infected adults	*Increase systemic exposure of atazanavir or darunavir in combination w. other antiretroviral agents in treatment of HIV-1 infection*
Conjugated estrogens/ bazedoxifene	EU: Duavive US: Duavee	EU: 2014 US: 2013	*Treatment of oestrogen deficiency symptoms in postmenopausal women with a uterus*	**1)** Treatment of moderate to severe vasomotor symptoms associated with menopause ***2)*** *Prevention of postmenopausal osteoporosis*
Daclatasvir	Daklinza	EU: 2014 US: 2015	In combination with other medicines for treatment of chronic hepatitis C virus infection	For use with sofosbuvir for the treatment of chronic HCV genotype 3 infection
DTG + 3TC/ ABC[b]	Triumeq	EU: 2014 US: 2014	Treatment of HIV infection	*Treatment of HIV-1 infection*
Edoxaban	EU: Lixiana US: Savaysa	EU: 2015 US: 2015	**1)** Prevention of stroke and systemic embolism in patients with nonvalvular atrial fibrillation **2)** Treatment of DVT and PE, and prevention of recurrent DVT and PE	**1)** Reduction of risk of stroke and systemic embolism in patients with nonvalvular atrial fibrillation **2)** Treatment of DVT and PE following initial therapy with a parenteral anticoagulant
Elvitegravir[c]	Vitekta	EU: 2013 US: 2014	Coadministered with ritonavir-boosted protease inhibitor and with other antiretroviral agents, for the treatment of HIV-1 infection in adults	*(…) in combination with an HIV protease inhibitor coadministered with ritonavir and other antiretroviral drug(s), for treatment of HIV-1 infection in adults*
Ivabradine	EU: Corlentor US: Corlanor	EU: 2005 US: 2015	***1)*** *Treatment of chronic stable angina pectoris* ***2)*** *Treatment of chronic HF*	Indicated to reduce the risk of hospitalization for worsening HF in patients with chronic HF
Lomitapide	EU: Lojuxta US: Juxtapid	EU: 2013 US: 2012	Adjunct to a low-fat diet and other lipid-lowering medicinal products in adult patients with HoFH	*Adjunct to a low-fat diet and other lipid-lowering treatments in patients with HoFH*
Netupitant / palonosetron	Akynzeo	EU: 2015 US: 2014	**1)** Nausea and vomiting associated with highly emetogenic cisplatin-based cancer chemotherapy **2)** Nausea and vomiting associated with moderately emetogenic cancer chemotherapy	*Prevention of acute and delayed nausea and vomiting associated with initial and repeat courses of cancer chemotherapy, including, but not limited to, highly emetogenic chemotherapy*

Novel therapeutic drug		Year of approval	Initially approved indication(s) - abbreviated[a]	
INN	**Brand name**		**EU**	**US**
Patiromer	Veltassa	EU: 2017 US: 2015	*Treatment of hyperkalaemia*	Treatment of hyperkalemia
Peginterferon beta-1a	Plegridy	EU: 2014 US: 2014	Treatment of relapsing remitting MS	Treatment of patients with relapsing forms of MS
Peramivir	US: Rapivab	EU: N/A US: 2014	*Not authorized*	Treatment of acute uncomplicated influenza
Pimavanserin	US: Nuplazid	EU: N/A US: 2016	*Not authorized*	Treatment of hallucinations and delusions associated with Parkinson's disease psychosis
Sacubitril/valsartan	Entresto	EU: 2015 US: 2015	Treatment of symptomatic chronic heart failure with reduced ejection fraction	Reduction of risk of CV death and hospitalization for heart failure in patients with chronic heart failure and reduced ejection fraction
Selexipag	Uptravi	EU: 2016 US: 2015	Long-term treatment of PAH in adult patients with WHO functional class II–III, as combination, or monotherapy	*Treatment of PAH (WHO Group I) to delay disease progression and reduce risk of hospitalization for PAH*
Sucroferric oxyhydroxide	Velphoro	EU: 2014 US: 2013	Control of serum phosphorus levels in adult chronic kidney disease patients on dialysis	*Indicated for the control of serum phosphorus levels in patients with chronic kidney disease on dialysis*
Susoctocog alfa	Obizur	EU: 2015 US: 2014	Treatment of bleeding episodes in patients with acquired haemophilia	*Indicated for the treatment of bleeding episodes in adults with acquired hemophilia A*
Teriflunomide	Aubagio	EU: 2013 US: 2012	*Treatment of relapsing remitting MS*	Treatment of relapsing forms of MS
Vedolizumab	Entyvio	EU: 2014 US: 2014	**1)** Moderate to severe ulcerative colitis ***2)*** *Mod. to severely active Crohn's disease*	**1)** Moderately to severely active ulcerative colitis ***2)*** *Mod. to severely active Crohn's disease*
Vorapaxar[d]	Zontivity	EU: 2015 US: 2014	Reduction of atherothrombotic events in patients w. history of MI	Reduction of thrombotic CV events in patients w. history of MI or peripheral arterial disease

[a] Abbreviations: AIDS – Acquired immune deficiency syndrome; cIAI - Complicated intra-abdominal infections; COPD - chronic obstructive pulmonary disease; CV – Cardiovascular; cUTI - Complicated urinary tract infections; DVT - Deep vein thrombosis; HIV - Human immunodeficiency virus; HF – Heart failure; HoFH - Homozygous familial hypercholesterolaemia; MI - Myocardial infarction; MS - Multiple sclerosis; PAH - Pulmonary arterial hypertension; PE - Pulmonary embolism. [b] abacavir / dolutegravir / lamivudine. [c] EU: Withdrawn. US: Discontinued. [d] EU: Withdrawn

Table S1 – Indications approved for novel therapeutic drugs based on a single pivotal trial

Novel therapeutic drugs (excluding oncology and orphan drugs) for which one or more indications were approved based on a single pivotal trial by FDA CDER and/or the EMA between 2012 and 2016. For combination products, the New Active Substance or New Molecular Entity component is underscored.

GREY shading and *italics* signify that the product or indication is either not authorized or does not fulfill the study selection criteria in the region in question.

BLUE and **RED** shadings indicate EU and US indications, respectively, that fulfilled the inclusion criteria.