EvaluatePharma®

Orphan Drug Report 2015

3rd Edition – October 2015

Evaluate®

Welcome to the EvaluatePharma® Orphan Drug Report 2015

The third edition of EvaluatePharma's Orphan Drug Report brings together many of our analyses to provide top-level insight, from the world's financial markets, into the expected performance of the orphan drug market between now and 2020. Based on EvaluatePharma's coverage of over 5,000 of the world's leading pharmaceutical and biotech companies, the Orphan Drug Report 2015 highlights trends in prescription sales for orphan vs. non-orphan drugs, orphan designation analysis in the USA and Europe, product and company performance, phase III R&D spend and return on investment. Additional copies are available at: www.evaluategroup.com/orphandrug2015

About EvaluatePharma

Since 1996 EvaluatePharma has been providing senior decision makers within the pharmaceutical industry with an essential service that models the sector from the viewpoint of the world's financial markets.

EvaluatePharma's forward looking view of the market is hugely influential as it displays the consensus of expectations, which influence company stock market valuations. The forecasts of equity analysts reveal their perspectives on individual company performance, industry trends and the macro economic environment.

EvaluatePharma has captured the consensus forecasts of equity analysts and seamlessly integrated them with the historic results, as reported by companies. From this comprehensive view of the industry, its past and expected future performance emerges and can be analysed using EvaluatePharma. Analyses range from total market trends and therapeutic overviews to individual company performance and product progress.

Whatever your view on the future of the industry, EvaluatePharma is the essential guide to value in the pharma and biotech sector.

EvaluatePharma's newest content set provides a fully integrated, single source for USA sales, volume and pricing information. In addition, annual cost per patient for the top selling drugs as well as number of patients treated is calculated for you. The analysis combines USA government data sources and is fully transparent as to the specific sources and the methodology of our calculations.

Download our report Budget Busters: the shift to high-priced innovator drugs in the USA or visit www.evaluategroup.com/budgetbusters

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To find out more about this report and Evaluate's range of services please contact us:

North America: Tom Moore
Tel: +1 617 573 9454 / Email: thomas.moore@evaluategroup.com

Rest of the World: Will Hau
Tel: +44 (0)20 7377 0800 / Email: will.hau@evaluategroup.com

Japan: Hiroshi Yamazaki
Tel: + 81 (0)80 1164 4754 / Email: hiroshi.yamazaki@evaluategroup.com

For general questions: Christine Lindgren
Tel: +1 617 866 3906 / Email: christine.lindgren@evaluategroup.com

Overview

An orphan drug is a pharmaceutical product aimed at rare diseases or disorders. The development of orphan drugs has been financially incentivised through US law via the Orphan Drug Act of 1983. The National Organization for Rare Disorders (NORD), which was instrumental in establishing the Act, currently estimates 30 million Americans suffer from 7,000 rare diseases. Prior to the 1983 Act, 38 orphan drugs were approved. The success of the original Orphan Drug Act in the US led to it being adopted in other key markets, most notably in Japan in 1993 and in the European Union in 2000.

Rare Disease Patient Populations are Defined in Law as:

- USA: <200,000 patients (<6.37 in 10,000, based on US population of 314m)
- EU: <5 in 10,000 (<250,000 patients, based on EU population of 506m)
- Japan: <50,000 patients (<4 in 10,000 based on Japan population of 128m)

Financial Incentives by Law Include:

Market Exclusivity

- USA: 7 Years of marketing exclusivity from approval; Note: Majority of orphan drugs have a compound patent beyond 7 years. The market exclusivity blocks 'same drug' recombinant products. E.g. Fabrazyme (Genzyme, now Sanofi) vs. Replagal (Transkaryotic, now Shire).'Same drug' exclusion can be overturned if clinically superior (mix of efficacy/ side effects). E.g. Rebif overturned Avonex's orphan drug exclusivity (7 MAR 2002)
- EU: 10 Years of marketing exclusivity from approval

Reduced R&D Costs

- USA: 50% Tax Credit on R&D Cost
- USA: R&D Grants for Phase I to Phase III Clinical Trials ($30m for each of fiscal years 2008-12)
- USA: User fees waived (FFDCA Section 526: Company WW Revenues <$50m)

Methodology on Classifying an Orphan Drug

We have identified all products that have orphan drug designations filed in the US, EU or Japan. These are available as part of the core EvaluatePharma service. To further enhance analysis, we have defined a clean 'Orphan' sub-set of products following a number of criteria including:

- First indication approved is for an orphan condition
- Products expected to generate more than 25% of sales from their orphan indications. This has led to the exclusion of drugs such as Avastin, Enbrel, Herceptin, Humira and Remicade, all of which have orphan designations for indications contributing less than 25% of sales
- Trial sizes, with smaller Phase III trials suggesting orphan status
- Drug pricing, higher prices were taken as an indicator of orphan status

All sales analysis in the report is based on this clean 'Orphan' sub-set of products.

Foreword

If anything sums up the current orphan drug market it is steady as she goes. This is now the third year that Evaluate has published its Orphan Drug Report and again there has been little change in the growth forecasts for this specialist end of drug development. However, the almost 12% annual growth seen in this sector is something that the general drug market could only dream about, with Evaluate forecasts putting its growth at half that at 5.9%.

> The one big change this year is that Celgene will have knocked Novartis off its perch as the world's biggest orphan drug company by sales in 2020. Celgene is set to achieve this solely on the back of the performance of its biggest drug Revlimid, which is used in 14 orphan drug indications in the US and Europe.

Indeed, large pharma groups finding orphan indications for some of their biggest sellers mean that seven of the 10 top companies by orphan indications are global majors, and the likes of AbbVie are trying to ensure their seat at the big table through acquisition. The Chicago-based group is now in the top 20 companies by orphan sales thanks to its acquisition of Pharmacyclics.

The Orphan Drug Report also reveals that worldwide orphan drug sales will reach $178bn by 2020. The proportion of orphan drugs in relation to the rest of the industry is also growing, with orphans set to account for just over 20% of all prescription drug sales by 2020, up slightly from the 19% Evaluate reported last year.

The continued health of the orphan drug market is in stark contrast to some of the recent turbulence experienced by the wider drug industry.

The enduring appeal of orphan drugs remains grounded in a variety of factors including the lack of alternatives for patients, lower R&D costs, easily defined patient populations, and the prices that the drugs are able to command.

So far orphan drug developers have managed to defend the cost of these life-changing drugs, due to the relatively small patient populations they serve and the continued paucity of options for sufferers.

Foreword

> But as the number of orphan drugs increases – and the report shows the number of US orphan drug designations increased by 12% to 291 in 2014 and rose an incredible 62% to 201 in Europe – it may become harder to justify prices.

As such, with increasing scrutiny over drug cost, particularly among lawmakers, and payers becoming more vocal in their desire to manage escalating drug bills, orphan drug developers will have to ensure that the drugs they produce continue to be innovative and can help reduce the overall healthcare budget.

Report Author:
Andreas Hadjivasiliou
Analyst, EvaluatePharma
Andreas.Hadjivasiliou@evaluategroup.com

Foreword by:
Lisa Urquhart
Editor, EP Vantage
lisau@epvantage.com

Analysis Highlights

- Worldwide orphan drug sales forecast to total $178bn (CAGR 2015 to 2020:+11.7%); Almost double overall prescription market growth
- Orphan drugs set to be 20.2% of worldwide prescription sales by 2020 (excluding generics)

- Median cost per patient differential 13.8 times higher for orphan drugs compared to non-orphan
- Celgene set to climb to number one position in orphan drug sales to 2020
- Vertex and Alexion set to march up the orphan drug sales ranking table
- Revlimid (lenalidomide) No.1 orphan drug in 2020
- Phase III orphan drug development cost half that of non-orphan, and potentially a quarter of the cost of non-orphan with US Tax Breaks
- Phase III drug development time for orphan drugs is no faster than non-orphan
- Orphan drug FDA approval time 10 months vs. 13 months for non-orphan
- Expected return on investment of Phase III/ Filed orphan drugs 1.14 times greater than non-orphan drugs
- Obeticholic acid (Intercept Pharmaceuticals) is most valuable R&D orphan drug
- Record year for FDA orphan designations in 2014 with 291; Record number of European designations too with 201; Japan designations up 7%
- Opdivo most promising new orphan drug approved by FDA in 2014
- 19.4% of EU orphan designations for ultra-rare diseases
- Non-Hodgkin Lymphoma (NHL) is indication with most filed orphan drug designations in EU

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Worldwide Orphan Drug Sales (2000-2020)

Worldwide orphan drug sales forecast to total $178bn (CAGR 2015 to 2020: +11.7%); Double overall Rx market growth; Orphan drugs set to be 20.2% of worldwide prescription sales by 2020 (excluding generics).

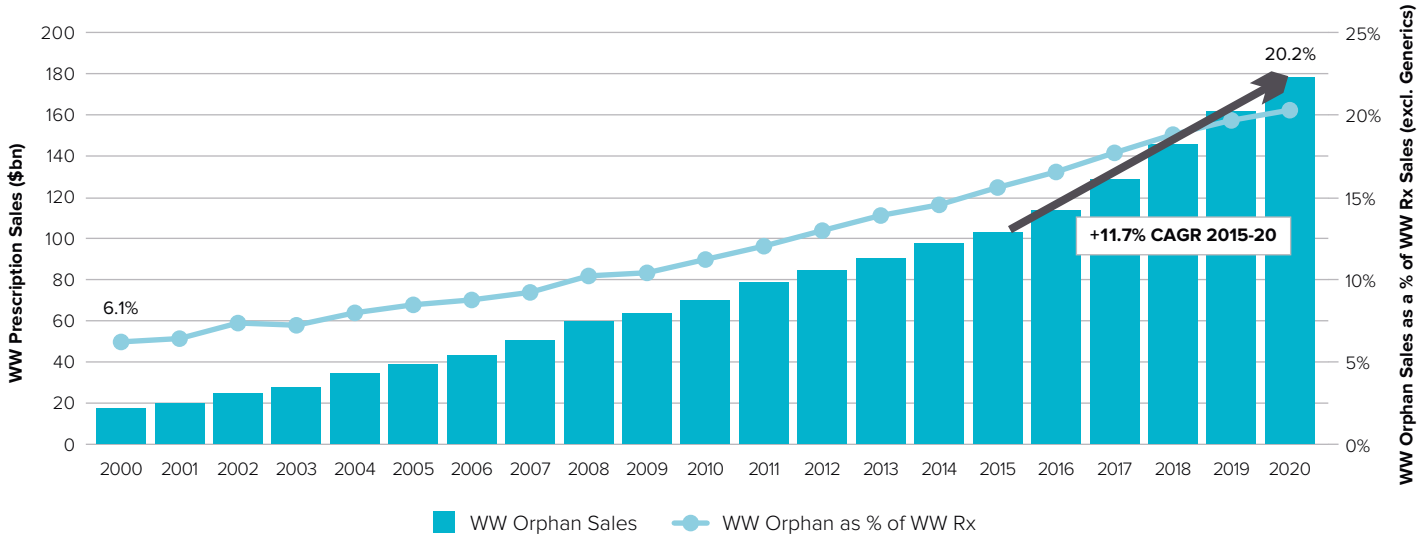
EvaluatePharma® finds that the market for orphan drugs, based on the consensus forecast for the leading 500 pharmaceutical and biotechnology companies, will grow by 11.7% per year (CAGR) between 2015 and 2020 to $178bn. The growth of the orphan drug market is almost double that of the overall prescription drug market, which is set to grow by 5.9% over the period 2015-2020.

Orphan drugs are set to account for 20.2% of global prescription sales in 2020, excluding generics, up from 6.1% in 2000.

In 2014 orphan drug sales increased 7.7% to $97bn vs. 2013, while non-orphan drug sales increased by 2.0% to $576bn.

Worldwide Orphan Drug Sales & Share of Prescription Drug Market (2000-2020)

Source: EvaluatePharma® 30 September 2015



Worldwide Orphan & Prescription Drug Sales (2006-2020)

Source: EvaluatePharma® 30 September 2015

	WW Prescription Sales ($bn)														
Year	2006	2007	2008	2009	2010	2011	2012	2013	2014	2015	2016	2017	2018	2019	2020
WW Orphan Drug Sales	44	51	60	63	70	79	84	90	97	102	114	129	145	161	178
Growth per Year		+15.4%	+19.3%	+5.1%	+10.7%	+12.7%	+6.2%	+7.2%	+7.7%	+5.6%	+10.9%	+13.1%	+12.9%	+11.1%	+10.3%
Orphan Sales as a % of Rx	8.7%	9.2%	10.1%	10.4%	11.2%	11.9%	12.9%	13.8%	14.4%	15.5%	16.4%	17.6%	18.6%	19.5%	20.2%
WW Non-Orphan Drug Sales	458	502	537	549	557	584	566	564	576	559	578	602	634	666	701
Growth per Year		+9.6%	+6.9%	+2.3%	+1.5%	+4.7%	-3.0%	-0.3%	+2.0%	-2.9%	+3.5%	+4.2%	+5.4%	+4.9%	+5.4%
WW Prescription (Rx) (less Generics)	502	553	597	612	628	663	650	654	673	661	692	731	779	827	879
Growth per Year		+10.1%	+8.0%	+2.6%	+2.5%	+5.6%	-1.9%	+0.7%	+2.8%	-1.7%	+4.6%	+5.6%	+6.7%	+6.1%	+6.3%

WW Orphan Drug Market CAGR 15-20 +11.7%
WW Non-Orphan Drug Market CAGR 15-20 +4.7%
WW Prescription (Rx) excluding Generics CAGR 15-20 +5.9%

Note: Industry sales based on pharmaceutical and biotech companies.

Sales to 2014 based on company reported sales data. Sales forecasts to 2020 based on a consensus of leading equity analysts' estimates for company product sales and segmental sales.

All sales analysis based on EvaluatePharma®'s clean 'Orphan' sub-set of products, as defined in the Overview section.

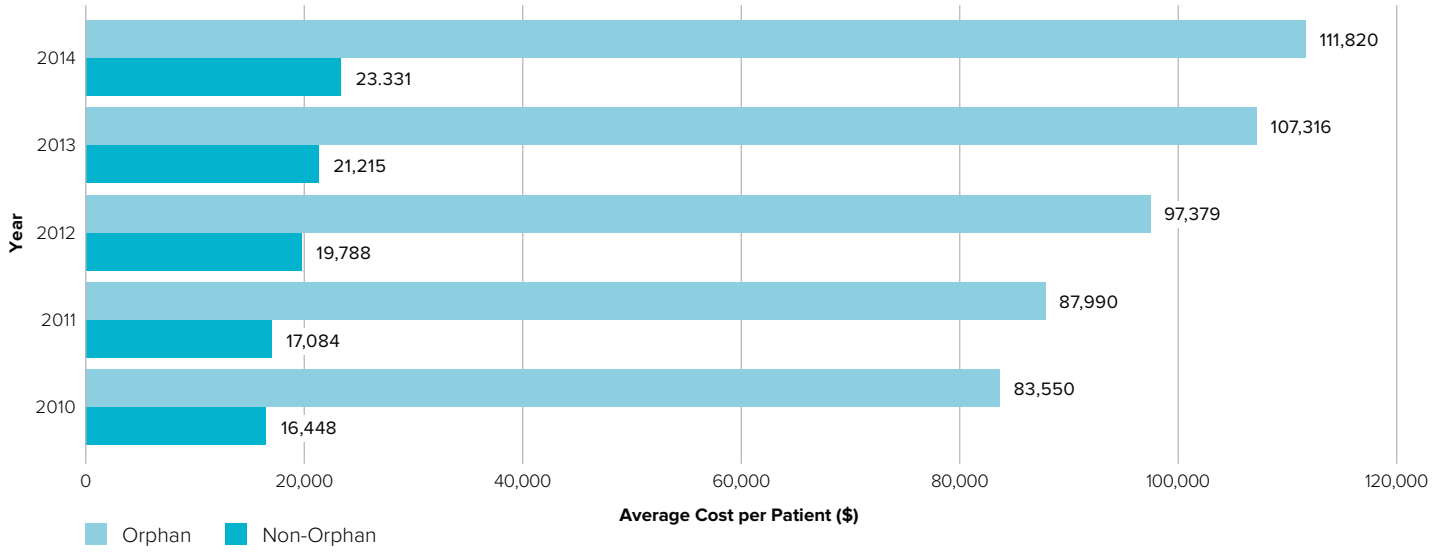
Top 100 USA Drug Cost per Patient per Year 2010-2014

Average orphan drug cost to patients $111,820 in 2014; Median orphan drug cost $66,057.

EvaluatePharma® estimates that the average cost per patient per year in 2014 for an orphan drug was $111,820 versus $23,331 for a non-orphan drug. The average and median drug price has increased year on year for both orphan and non-orphan drugs since 2010. The median price of orphan and non-orphan drugs has increased by a factor of 1.8 and 1.7 since 2010 respectively.

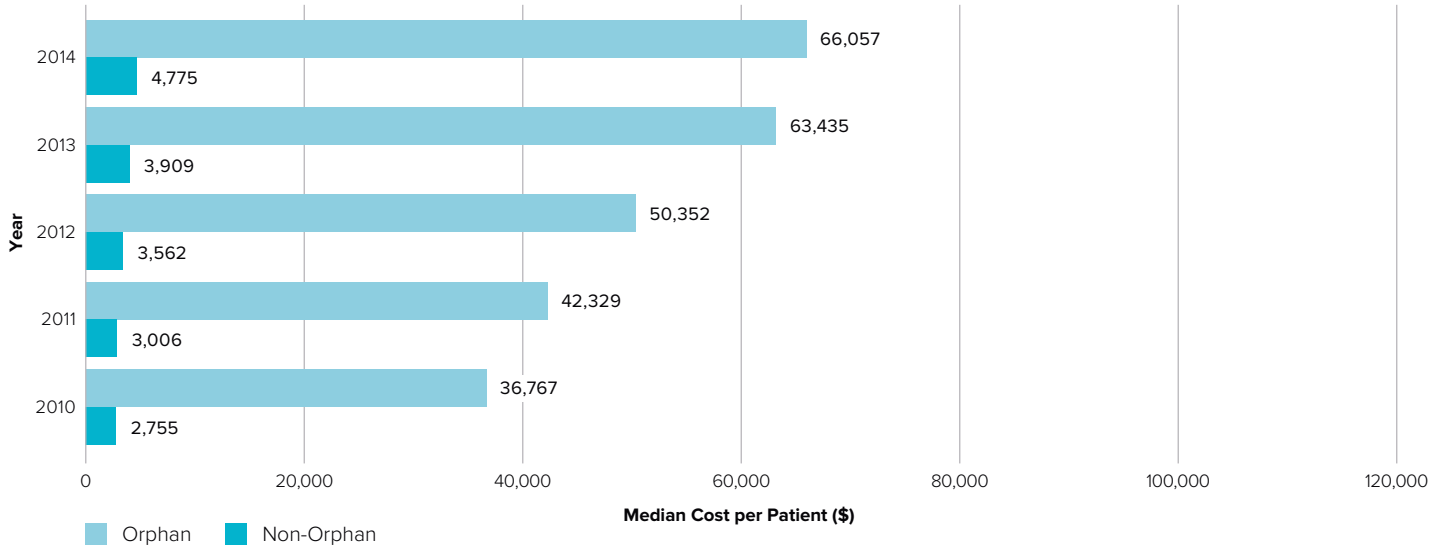
Average Cost per Patient per Year 2010-2014

Source: EvaluatePharma® 30 September 2015



Median Cost per Patient per Year 2010-2014

Source: EvaluatePharma® 30 September 2015



Top 100 Orphan and Top 100 Non-Orphan USA Drugs by Sales Average and Median Cost per Patient per Year 2010-2014

Source: EvaluatePharma® 30 September 2015

Average Cost per Patient ($) per year	2010	2011	2012	2013	2014		CAGR
Orphan	83,550	87,990	97,379	107,316	111,820		7.6%
Growth per Year		5.3%	10.7%	10.2%	4.2%		
Median price	36,767	42,329	50,352	63,435	66,057		
Non-orphan	16,448	17,084	19,788	21,215	23,331		9.1%
Growth per Year		3.9%	15.8%	7.2%	10.0%		
Median price	2,755	3,006	3,562	3,909	4,775		
Median Price Differential (orphan/non-orphan)	13.3	14.1	14.1	16.2	13.8		
					Median price increase 2010-2014:		
					Orphan:	1.80	
					Non-orphan:	1.73	

Note: All sales analysis based on EvaluatePharma®'s clean 'Orphan' sub-set of products, as defined in the Overview section.

Cost per patient is an estimate for the retail cost of a drug to a patient, for a given year, based on a 100% compliance to the treatment guidelines outlined in the FDA label. Does not include off-invoice discounts. The Top 100 orphan and non drugs were ranked by USA sales for 2014.

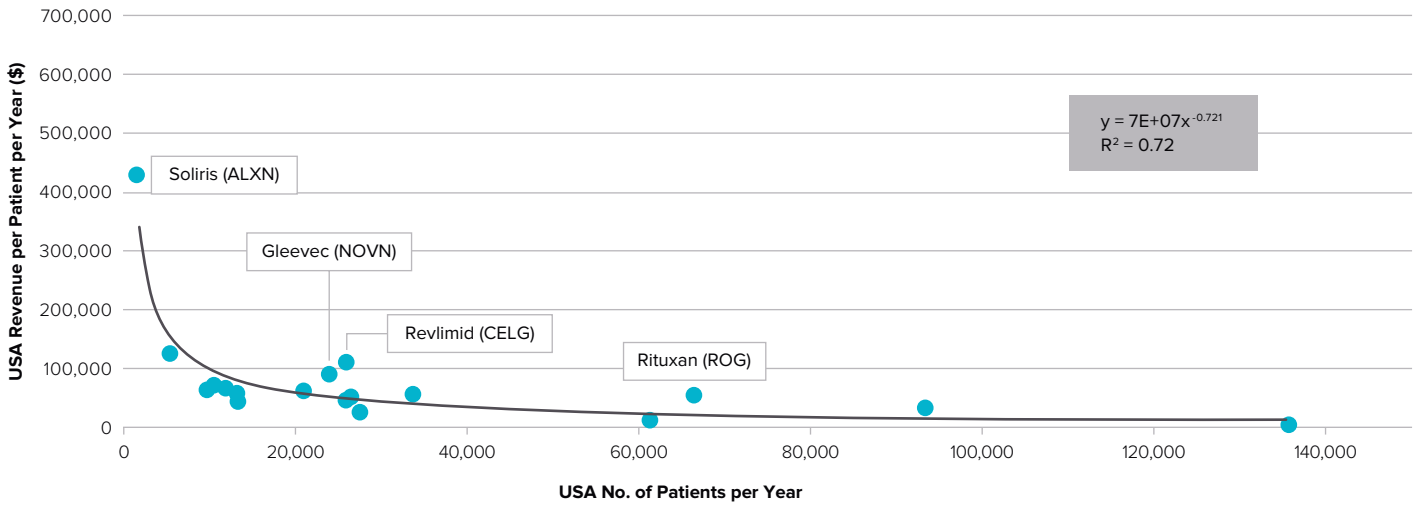
2014: USA Revenue per Patient per Year for Top 20 Selling Orphan Drugs

Soliris Highest Revenue Orphan Drug in 2014; Revenue per Patient and Number of Patients Treated Correlated; Orphan Drugs That Treated Fewer Than 10,000 Patients: Closer Correlation of Drug Price and Patient Numbers.

EvaluatePharma® finds that revenue per patient for the Top 20 USA selling orphan drugs is correlated (R^2 = 0.72) to the number of patients treated in 2014. A similar analysis of the Top 10 selling orphan drugs that treated fewer than 10,000 patients confirms a closer correlation (R^2 = 0.77). This analysis confirms industry perceptions that smaller patient groups allow a pricing premium to be achieved versus non orphans. Products such as Gleevec support the notion of an innovation premium for drugs that create a step change in treatment options and therapy outcomes. Soliris confirms the pricing power resulting from indications with the fewest number of patients.

Top 20 USA Orphan Drugs in 2014 by Sales; Revenue per Patient vs. No. of Patients Treated

Source: EvaluatePharma® 30 September 2015



USA Top 10 Selling Orphan Drugs in 2014 by Sales

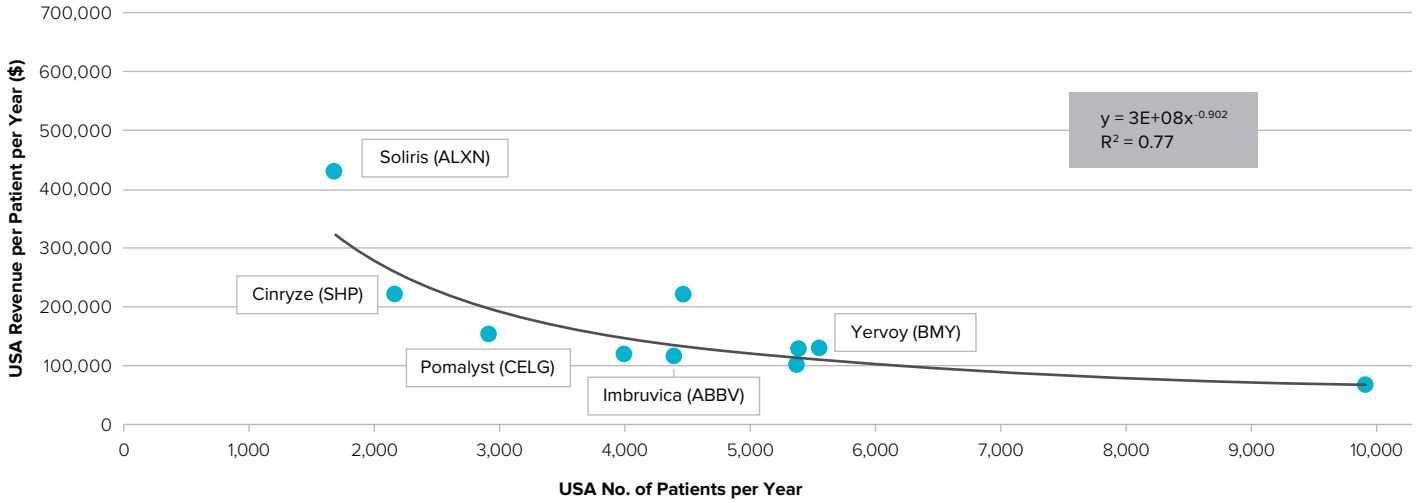
Source: EvaluatePharma® 30 September 2015

Rank	Product	Generic Name	Company	USA Sales ($m) 2014	Revenues per Patient 2014*	No. of Patients 2014
1.	**Rituxan**	rituximab	Roche	3,646	54,780	66,565
2.	**Copaxone**	glatiramer acetate	Teva Pharmaceutical Industries	3,113	33,309	93,458
3.	**Revlimid**	lenalidomide	Celgene	2,916	112,294	25,965
4.	**Gleevec**	imatinib mesylate	Novartis	2,170	90,634	23,943
5.	**Avonex**	interferon beta-1a	Biogen	1,957	57,932	33,781
6.	**Velcade**	bortezomib	Takeda	1,396	52,838	26,414
7.	**Rebif**	interferon beta-1a	Merck KGaA	1,290	61,631	20,924
8.	**Alimta**	pemetrexed disodium	Eli Lilly	1,230	47,378	25,951
9.	**Advate**	factor VIII (procoagulant)	Baxalta	985	220,839	4,460
10.	**Afinitor**	everolimus	Novartis	805	66,390	12,125

2014: USA Revenue per Patient per Year for Top 20 Selling Orphan Drugs

Top 10 USA Orphan Drugs in 2014 by Sales (fewer than 10,00 patients treated); Revenue per Patient vs. No. of Patients Treated

Source: EvaluatePharma® 30 September 2015



Note: Sales represent company reported sales where available, otherwise based on an average of equity analyst estimates.

USA sales represent sales for all indications.

EvaluatePharma® analysed the Top 10 selling USA drugs which treated fewer than 10,000 patients in 2014.

*Revenues per patient: An estimate of the dollar ($) revenues per year received, by a company, per patient for a drug in the USA market. This takes into account the cost per patient (average mg per year multiplied by the cost per mg), off-invoice discount and patient compliance.

All sales analysis based on EvaluatePharma®'s clean 'Orphan' sub-set of products, as defined in the Overview section.

Worldwide Orphan Drug Sales in 2020: Top 20 Companies

Celgene number one in orphan drug sales in 2020 marginally ahead from Novartis and Bristol-Myers Squibb. Alexion and Vertex set to march up the orphan sales ranking table.

EvaluatePharma® finds that Celgene will overtake Novartis as the world's number one orphan drug company in 2020, climbing 2 places, and pushing Novartis down to number two.

The top four companies in the table are ranked within $240m of each other. One product contributes the majority of sales for three of the four; Celgene (Revlimid), BMY (Opdivo) and Roche (Rituxan).

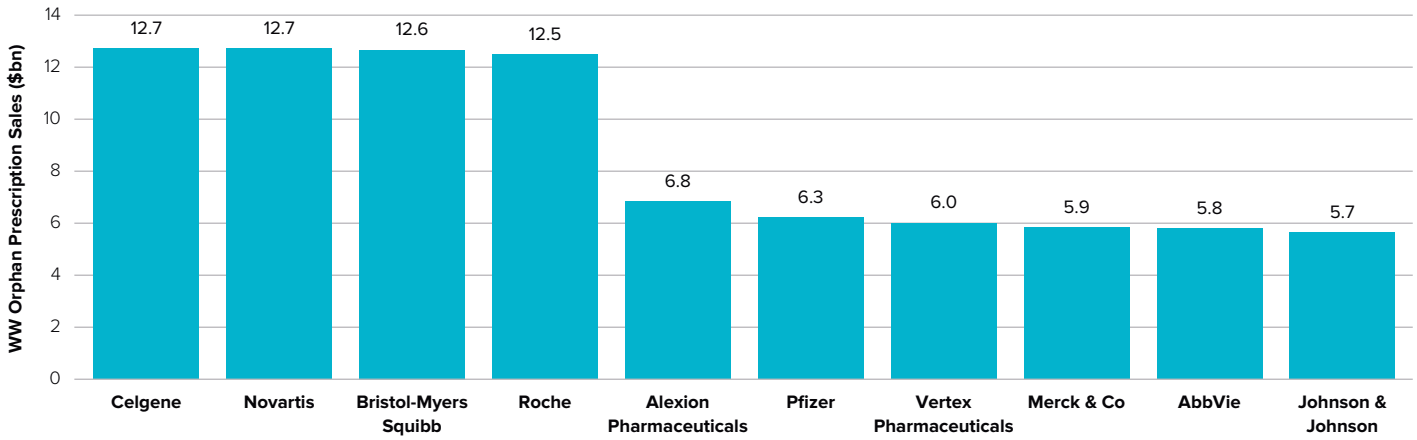
AbbVie joins the top 20 following its acquisition of Pharmacyclics.

Seven of the Top 10 companies by orphan drug sales in 2020 are forecast to be Global Majors.

The Top 5 companies in 2020 are forecast to account for almost one third (32.2%) of the orphan drug market.

WW Orphan Prescription Drug Sales in 2020: Top 10 Companies

Source: EvaluatePharma® 30 September 2015



Worldwide Orphan Drug Sales (2014-2020): Top 20 Companies & Total Market

Source: EvaluatePharma® 30 September 2015

		WW Orphan Sales ($bn)			WW Orphan Market Share			Rank
Rank	Company	2014	2020	% CAGR 14-20	2014	2020	Chg. (+/-)	Chg. (+/-)
1.	**Celgene**	**6.6**	**12.7**	12%	6.8%	7.2%	+0.4%	+2
2.	**Novartis**	**11.8**	**12.7**	1%	12.2%	7.1%	-5.1%	-1
3.	**Bristol-Myers Squibb**	**2.9**	**12.6**	28%	2.9%	7.1%	+4.2%	+9
4.	**Roche**	**9.7**	**12.5**	4%	10.0%	7.0%	-2.9%	-2
5.	**Alexion Pharmaceuticals**	**2.2**	**6.8**	21%	2.3%	3.8%	+1.5%	+11
6.	**Pfizer**	**5.3**	**6.3**	3%	5.5%	3.5%	-2.0%	-2
7.	**Vertex Pharmaceuticals**	**0.5**	**6.0**	53%	0.5%	3.4%	+2.9%	+22
8.	**Merck & Co**	**1.1**	**5.9**	32%	1.1%	3.3%	+2.2%	+15
9.	**AbbVie**	**0.2**	**5.8**	73%	0.2%	3.3%	+3.0%	+31
10.	**Johnson & Johnson**	**2.0**	**5.7**	19%	2.1%	3.2%	+1.1%	+8
11.	**Shire**	**2.5**	**4.8**	12%	2.5%	2.7%	+0.2%	+3
12.	**Baxalta**	**3.9**	**4.7**	3%	4.0%	2.7%	-1.3%	-5
13.	**Sanofi**	**3.7**	**4.5**	3%	3.8%	2.5%	-1.3%	-5
14.	**Eli Lilly**	**3.1**	**4.0**	4%	3.2%	2.3%	-0.9%	-4
15.	**Novo Nordisk**	**2.8**	**3.5**	4%	2.9%	2.0%	-0.9%	-2

Top 16-20 continued over...

Worldwide Orphan Drug Sales in 2020: Top 20 Companies

Rank	Company	WW Orphan Sales ($bn) 2014	2020	% CAGR 14-20	WW Orphan Market Share 2014	2020	Chg. (+/-)	Rank Chg. (+/-)
16.	**Amgen**	**2.0**	**3.1**	8%	2.0%	1.8%	-0.3%	+3
17.	**Actelion**	**2.1**	**3.1**	7%	2.1%	1.8%	-0.4%	-
18.	**Bayer**	**3.9**	**3.0**	-4%	4.0%	1.7%	-2.3%	-12
19.	**BioMarin Pharmaceutical**	**0.6**	**2.6**	26%	0.7%	1.4%	+0.8%	+9
20.	**Biogen**	**3.1**	**2.5**	-4%	3.2%	1.4%	-1.9%	-11
	Total Top 20	**69.9**	**122.8**	+9.9%	72.0%	69.1%	-3.0%	
	Other	**27.1**	**55.0**	+12.5%	28.0%	30.9%		
	Total	**97.0**	**177.8**	+10.6%	100.0%	100.0%		

Note: All sales analysis based on EvaluatePharma®'s clean 'Orphan' sub-set of products, as defined in the Overview section.

2020: Top 20 Selling Orphan Drugs in the World

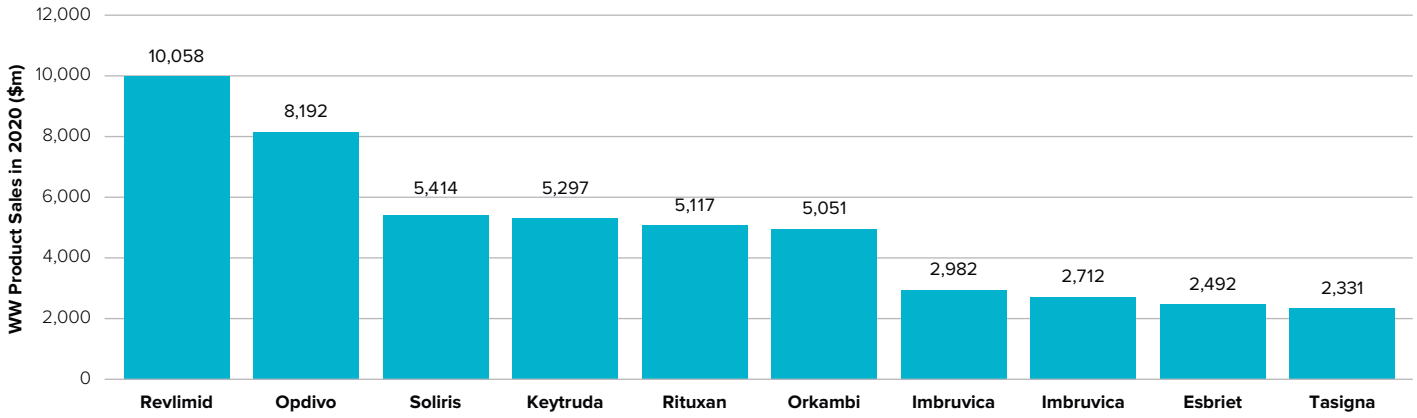
Revlimid (lenalidomide) No.1 orphan drug in 2020.

EvaluatePharma® finds that Revlimid is the world's largest orphan drug in 2020, with sales of $10bn for all indications. Revlimid from Celgene was first approved in December 2005 for the orphan treatment of myelodysplastic syndrome. Revlimid is also approved for the orphan indications Non-Hodgkin lymphoma and multiple myeloma and remains in development for a number of other orphan conditions.

Bristol-Myers Squibb's Opdivo approved for melanoma and designated for Hodgkin lymphoma, small cell lung cancer, hepatoma and glioblastoma is set to be a distant second with $8.2bn in worldwide sales.

Top 10 Selling Orphan Drugs in 2020 by Worldwide Sales

Source: EvaluatePharma® 30 September 2015



Worldwide Top 20 Selling Orphan Drugs in 2020

Source: EvaluatePharma® 30 September 2015

Rank	Product	Generic Name	Company	Phase (Current)	Pharmacological Class	WW Product Sales ($m) 2014	2020	CAGR
1.	**Revlimid**	lenalidomide	Celgene	Marketed	Immunomodulator	4,980	**10,058**	+12%
2.	**Opdivo**	nivolumab	Bristol-Myers Squibb	Marketed	Anti-programmed death-1 (PD-1) MAb	6	**8,192**	+233%
3.	**Soliris**	eculizumab	Alexion Pharmaceuticals	Marketed	Anti-complement factor C5 MAb	2,234	**5,414**	+16%
4.	**Keytruda**	pembrolizumab	Merck & Co	Marketed	Anti-programmed death-1 (PD-1) MAb	55	**5,297**	+114%
5.	**Rituxan**	rituximab	Roche	Marketed	Anti-CD20 MAb	7,547	**5,117**	-6%
6.	**Orkambi**	lumacaftor; ivacaftor	Vertex Pharmaceuticals	Marketed	Cystic fibrosis transmembrane conductance regulator (CFTR) corrector	-	**5,051**	n/a
7.	**Imbruvica**	ibrutinib	AbbVie	Marketed	Bruton's tyrosine kinase (BTK) inhibitor	-	**2,982**	n/a
8.	**Imbruvica**	ibrutinib	Johnson & Johnson	Marketed	Bruton's tyrosine kinase (BTK) inhibitor	55	**2,712**	+91%
9.	**Esbriet**	Pirfenidone	Roche	Marketed	Tumour necrosis factor alpha (TNFα) & transforming growth factor-beta (TGF-β) inhibitor	48	**2,492**	+93%
10.	**Tasigna**	nilotinib hydrochloride monohydrate	Novartis	Marketed	BCR-ABL tyrosine kinase inhibitor	1,529	**2,331**	+7%

Top 11-20 continued over...

2020: Top 20 Selling Orphan Drugs in the World

Rank	Product	Generic Name	Company	Phase (Current)	Pharmacological Class	WW Product Sales ($m) 2014	2020	CAGR
11.	**Pomalyst**	pomalidomide	Celgene	Marketed	Immunomodulator	680	**2,060**	+20%
12.	**Alimta**	pemetrexed disodium	Eli Lilly	Marketed	Thymidylate synthase inhibitor	2,792	**2,019**	-5%
13.	**Gazyva**	obinutuzumab	Roche	Marketed	Anti-CD20 MAb	54	**1,932**	+82%
14.	**Advate**	factor VIII (procoagulant)	Baxalta	Marketed	Factor VIII	2,348	**1,918**	-3%
15.	**Kyprolis**	carfilzomib	Amgen	Marketed	Proteasome inhibitor	331	**1,857**	+33%
16.	**Obeticholic acid**	obeticholic acid	Intercept Pharmaceuticals	Filed	Farnesoid X receptor (FXR) agonist	-	**1,827**	n/a
17.	**Yervoy**	ipilimumab	Bristol-Myers Squibb	Marketed	Anti-cytotoxic T lymphocyte associated protein 4 (CTLA4) MAb	1,308	**1,723**	+5%
18.	**Ofev***	nintedanib	Boehringer Ingelheim	Marketed	Tyrosine kinase inhibitor	5	**1,674**	+164%
19.	**Cyramza**	ramucirumab	Eli Lilly	Marketed	Anti-VEGF-2 MAb	76	**1,655**	+67%
20.	**Sprycel**	dasatinib	Bristol-Myers Squibb	Marketed	Tyrosine kinase inhibitor	1,493	**1,646**	+2%
	Other					**71,487**	**109,870**	+7%
	Total					**97,026**	**177,827**	+10.6%

Note: * Forecast based on a single broker model.

Sales represent company reported sales where available, otherwise based on an average of equity analyst estimates.

Worldwide sales represent sales for all indications.

All sales analysis based on EvaluatePharma®'s clean 'Orphan' sub-set of products, as defined in the Overview section.

Phase III Trial Size & Approval Time: Orphan vs. Non-Orphan

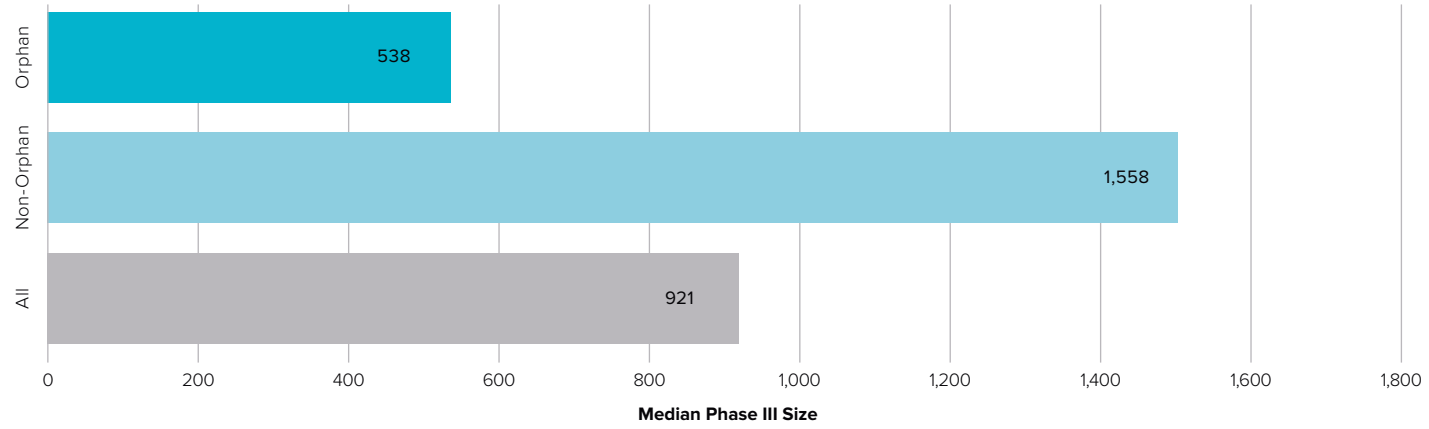
Phase III orphan drug development cost half that of non-orphan, potentially a quarter with US tax breaks; Phase III drug development time for orphan is no quicker than non-orphan.

Orphan drug FDA approval time quicker at 10.1 months vs. 12.9 months for non-orphan.

EvaluatePharma® estimates that the average phase III clinical trial cost for an orphan drug is roughly half that of non-orphan drugs at $103m, vs. $193m for a non-orphan. In addition, with 50% US tax credit, available via the Orphan Drug Act, the potential cost could be approximately a quarter of non-orphan at $51m vs. $193m for non-orphans. Orphan drugs required a median 538 patients for phase III trials, versus a median 1,558 patients for non-orphan drugs. The average phase III trial sizes came in at 761 patients for orphan drugs versus 3,549 for non-orphans. EvaluatePharma® found that there was no statistically significant difference in phase III trial length, at a median of 2.88 years. Median FDA approval times were 10 months for orphan vs. 13 months for non-orphans, due to orphans tending to receive Priority Review.

Phase III Trials Sizes: Orphan vs. Non-Orphan

Source: EvaluatePharma® 30 September 2015

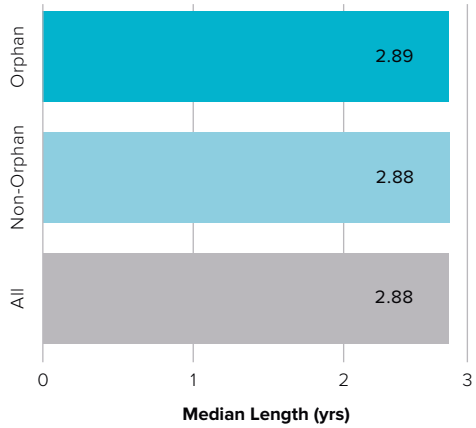


Average Phase III Trials Sizes (All New Drug Products Entering Phase III from 1 JAN 2000)

Source: EvaluatePharma® 30 September 2015

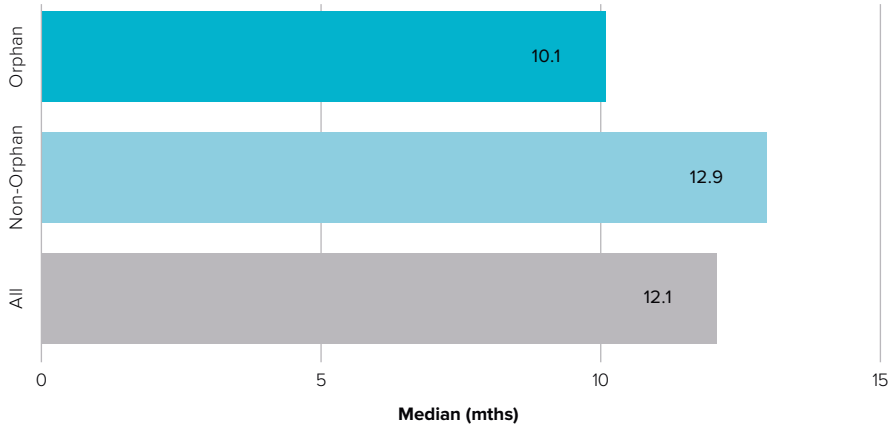
Product Type	Phase III Trial Size					Phase III Cost ($m) Estimated*			Potential 50% US Tax Credit	
	Median	Average	No. of Products (n=)	Total Patients	% of	Median	Average	Total	Median	Average
Orphan	538	761	466	354,705	10%	99	103	47,929	49	51
Non-Orphan	1,558	3,549	952	3,378,809	90%	150	193	183,543	150	193
All	921	2,633	1,418	3,733,514	100%	127	163	231,472		
Orphan / Non-Orphan =	34.5%	21.4%	48.9%			65.7%	53.3%	26.1%	32.9%	26.7%

Phase III Trials Length (yrs): Orphan vs. Non-Orphan



FDA Approval Time: Orphan vs. Non-Orphan

Source: EvaluatePharma® 30 September 2015



Average Phase III & FDA Approval Times

Source: Provisional Data from EvaluatePharma®'s Forthcoming Success Rates & Clinical Trial Timelines – 30 September 2015

Product Type	Phase III Length (yrs)			FDA Filed to Approved (mths)		
	Median	Average	No. of Products (n=)	Median	Average	No. of Products (n=)
Orphan	2.89	3.64	193	10.09	10.45	229
Non-Orphan	2.88	3.31	437	12.90	12.63	530
All	2.88	3.41	630	12.13	11.97	759
Orphan / Non-Orphan =	+0.5%	+9.8%		-21.8%	-17.3%	

Note: Analysis based on Lead indication.

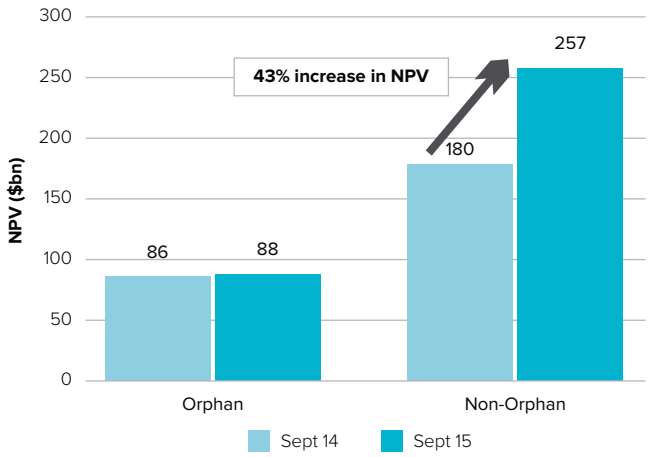
R&D Costs (PIII/ Filed) & Expected Investment Returns (NPV)

Expected return on investment of Phase III/ Filed orphan drugs 1.14 Times greater than non-orphan drugs; Phase III/ Filed orphan drugs are 23% of industry Phase III R&D spend and 26% of the value creation in the industry.

EvaluatePharma® finds that the current value, based on NPV, of pipeline phase III/ filed orphan drugs is $88bn versus $257bn for non-orphans. When looking at the industry's expected phase III costs to bring all products to market, orphan drugs cost $6.9bn versus $23.1bn for non-orphans. We have not assumed any US tax credits in the calculation. The main difference in cost is driven by the differing phase III trial sizes, with all current phase III/ filed orphans set to require a total of 44,357 patients for phase III trials, versus 426,951 patients for non-orphans.

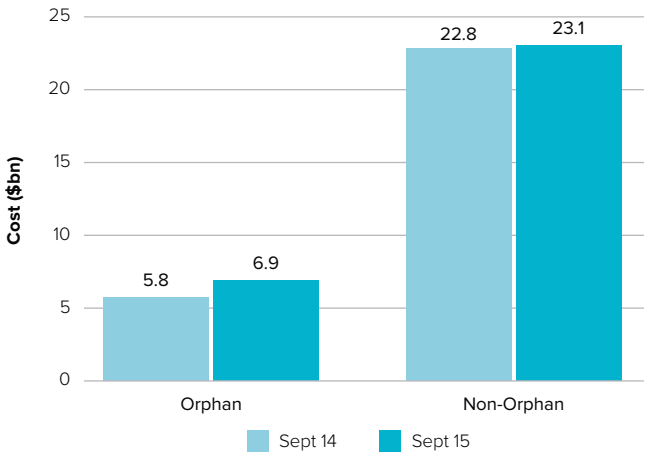
The current expected return on investment can be calculated taking the NPV divided by the phase III cost. The industry's investment return on orphan drugs is 1.14 compared to a non-orphan drug. The NPV for orphan assets has remained relatively constant compared to last year ($88bn vs $86bn) in contrast to non-orphan assets whose NPV increased by 43% to $257bn.

R&D (Phase III/Filed): NPV ($bn)

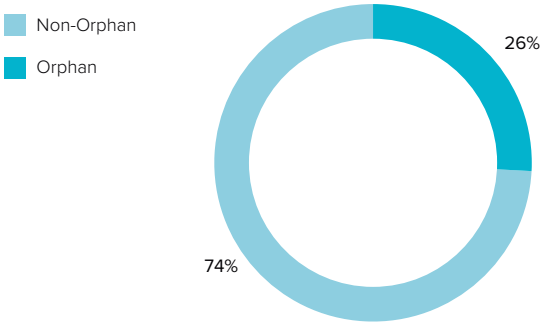


Phase III Cost ($bn)

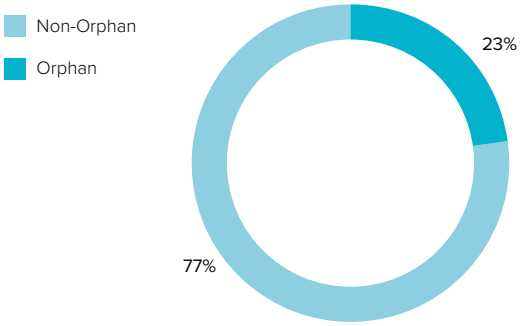
Source: EvaluatePharma® 30 September 2015



R&D NPV for Phase III/Filed ($bn)



Phase III R&D Costs ($bn)



R&D Costs (PIII/ Filed) & Expected Investment Returns (NPV)

Source: Provisional Data from EvaluatePharma®'s Forthcoming Success Rates & Clinical Trial Timelines – 30 September 2015

Value Creation (NPV) for NMEs
Phase III/ Filed with NPV

Phase III Clinical Trial Cost (estimated)
Available Current Phase III Costs

Expected Return
NPV minus Phase III Cost

Type	NPV ($bn) 2014	NPV ($bn) 2015	As a %	Avg. Product NPV ($m)	No. of Products (n=)	No. of Patients	Phase III Cost ($bn)	As a %	No. of Products (n=)	Net Return NPV - Phase III Cost	NPV/ Phase III Cost
Orphan	86	88	26%	1,013	87	44,357	6.9	23%	69	+81.1	12.7
Non-Orphan	180	257	74%	1,477	174	426,951	23.1	77%	117	+233.9	11.1
Total	**266**	**345**	**100%**	**1,322**	261	**471,308**	**30.1**	**100%**	186	**+315.0**	**11.5**

Investment Return Relative to Non-Orphan 1.14

Note: The sample size for product NPVs (n=261) is greater than the estimates of phase III trial sizes (n=186). This is due to NPVs being split by licensing deals. Not all products have an estimate of Phase III trial costs, mainly due to the fact certain products entered Phase III prior to 1 JAN 2000 (the current starting date of EvaluatePharma®'s Success Rate Model).

Phase III trial costs have been estimated based on an algorithm using cost per patient per year.

2020: Top 20 Orphan R&D Products based on NPV

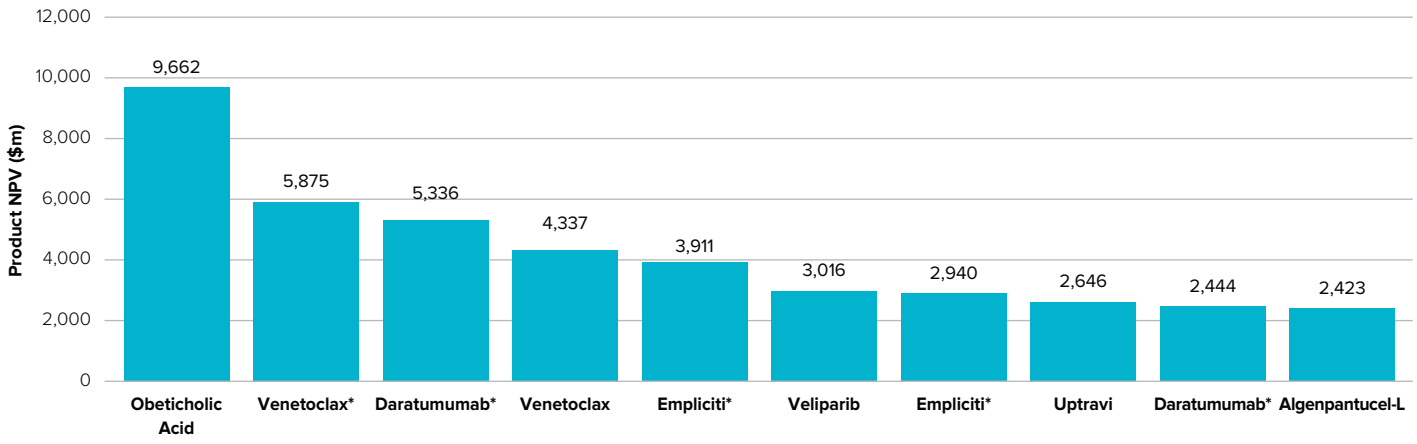
Obeticholic acid world's most valuable R&D orphan drug.

EvaluatePharma® finds that Intercept Pharmaceuticals's Farnesoid X receptor (FXR) agonist in development for Primary biliary cirrhosis (PBC), is the world's most promising R&D orphan drug, with an NPV of $9.7bn. 24% of the total orphan NPV is attributed to the top 3 ranked products.

Abbvie was the only company to have more than one product (Venetoclax, Empliciti and Veliparib) listed in the top 20 but will share certain markets for two of these with Roche and Bristol-Myers Squibb respectively.

Top 10 Orphan Drugs in 2020 (Phase III/Filed) by NPV

Source: EvaluatePharma® 30 September 2015



Worldwide Top 20 Orphan R&D Products based on NPV (Sales, NPV, PIII Cost & Expected Return)

Source: EvaluatePharma® 30 September 2015

Rank	Product	Company	Phase (Current)	Pharma Class	Sales ($m) 2020	WW NPV	R&D PIII Cost $m	Phase III Trial Size	Return NPV/PIII Cost	Strategy
1.	**Obeticholic acid**	Intercept Pharmaceuticals	Filed	Farnesoid X receptor (FXR) agonist	1,827	**9,662**	78	180	124	Organic
2.	**Venetoclax***	AbbVie	Phase III	B-cell lymphoma 2 (Bcl-2) inhibitor	1,176	**5,875**	128	802	46	Organic
3.	**Daratumumab***	Johnson & Johnson	Filed	Anti-CD38 MAb	1,166	**5,336**	250	2,470	21	In-licensed
4.	**Venetoclax***	Roche	Phase III	B-cell lymphoma 2 (Bcl-2) inhibitor	742	**4,337**	n/a	n/a	n/a	Company acquisition
5.	**Empliciti***	Bristol-Myers Squibb	Filed	Anti-signalling lymphocyte activation molecule (SLAMF7) MAb	779	**3,911**	217	2,140	18	In-licensed
6.	**Veliparib**	AbbVie	Phase III	Poly (ADP-ribose) polymerase (PARP) inhibitor	527	**3,016**	99	624	30	Organic
7.	**Empliciti***	AbbVie	Filed	Anti-signalling lymphocyte activation molecule (SLAMF7) MAb	375	**2,940**	n/a	n/a	n/a	Company acquisition
8.	**Uptravi**	Actelion	Filed	Prostacyclin receptor agonist	1,204	**2,646**	185	1,826	14	In-licensed
9.	**Daratumumab***	Genmab	Filed	Anti-CD38 MAb	-	**2,444**	n/a	n/a	n/a	Organic
10.	**Algenpantucel-L**	NewLink Genetics	Phase III	Cancer vaccine	663	**2,423**	101	1,002	24	Organic

Top 11-20 continued over...

2020: Top 20 Orphan R&D Products based on NPV

Rank	Product	Company	Phase (Current)	Pharma Class	Sales ($m) 2020	WW NPV	R&D PIII Cost $m	Phase III Trial Size	Return NPV/PIII Cost	Strategy
11.	**Drisapersen**	BioMarin Pharmaceutical	Filed	Muscular dystrophy antisense	652	**2,337**	29	67	80	Company acquisition
12.	**Austedo**	Teva Pharmaceutical Industries	Filed	Vesicular monoamine transporter 2 (VMAT2) inhibitor	549	**2,225**	91	209	25	Company acquisition
13.	**Patisiran**	Alnylam Pharmaceuticals	Phase III	Transthyretin RNAi therapeutic	434	**1,894**	87	200	22	Organic
14.	**NEOD001**	Prothena	Phase III	Anti-beta-amyloid (Abeta) MAb	397	**1,779**	102	236	17	Organic
15.	**CSL654**	CSL	Filed	Factor IX	386	**1,699**	77	178	22	Organic
16.	**Andexanet Alfa**	Portola Pharmaceuticals	Phase III	Factor Xa inhibitor antidote	256	**1,590**	119	413	13	Organic
17.	**Niraparib**	TESARO	Phase III	Poly (ADP-ribose) polymerase (PARP) inhibitor	499	**1,534**	142	490	11	In-licensed
18.	**Eteplirsen**	Sarepta Therapeutics	Filed	Muscular dystrophy antisense	544	**1,340**	69	160	19	Organic
10.	**Inotuzumab Ozogamicin**	Pfizer	Phase III	Anti-CD22 MAb-calicheamicin conjugate	267	**1,321**	107	369	12	Company acquisition
20.	**Duvelisib**	Infinity Pharmaceuticals	Phase III	Phosphatidylinositol 3-kinase (PI3K)-delta/gamma inhibitor	488	**1,319**	130	450	10	In-licensed
	Other				**7,127**	**28,462**				
	Total				**20,059**	**88,089**	**6,949**	**44,357**	**12.7**	
				vs. Non-Orphan:	**55,964**	**257,032**	**23,129**	**426,951**	**11.1**	

Note: Sales represent company reported sales where available, otherwise based on an average of equity analyst estimates.

Phase III trial costs represent an assumption on current phase III trial size for lead indication(s), derived from the enrolment size listed on clinicaltrials.gov via Evaluate Clinical Trials.

* Venetoclax, Empliciti and Daratumumab are presented based on NPV value to company retaining specific market rights. Phase III details are accounted for once for these products.

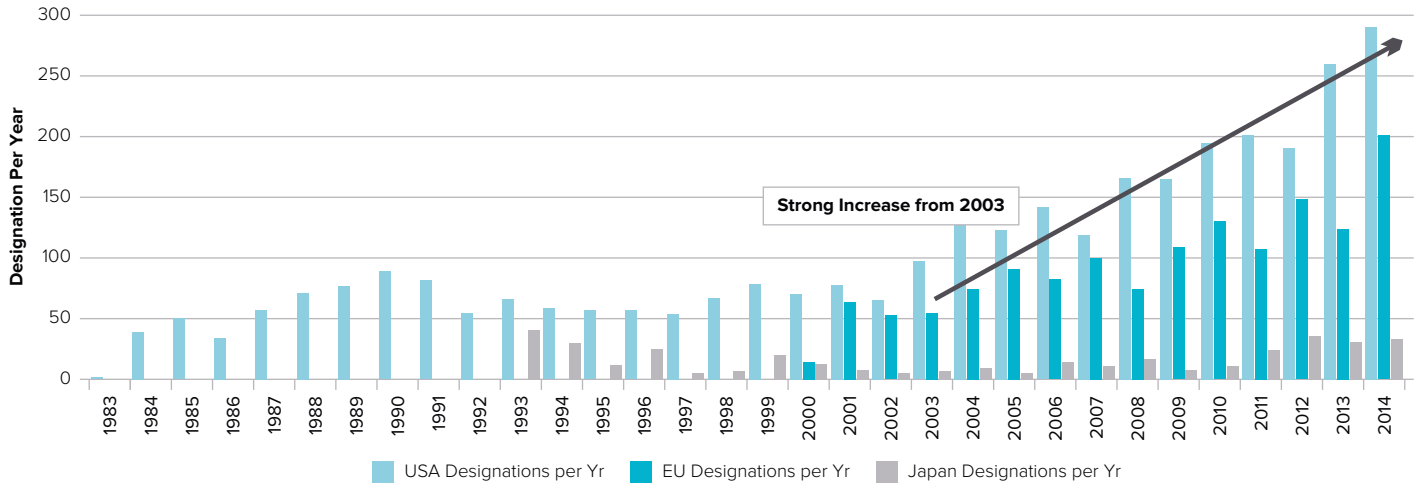
USA, EU & Japan Orphan Designations per Year & Cumulative (1983-2014)

FDA orphan designations increase 12% in 2014; European designations up 62%; Japan designations up 7%.

EvaluatePharma® found that the number of US orphan drug designations increased 12% in 2014 to 291, the highest number of designations seen so far, breaking the 2013 record of 260. European orphan designations rebounded strongly from a 2013 drop to post a 62% increase. Orphan designations in Japan increased by 7% to 32 designations.

USA, EU & Japan Designations per Year (1983-2014)

Source: EvaluatePharma® 30 September 2015



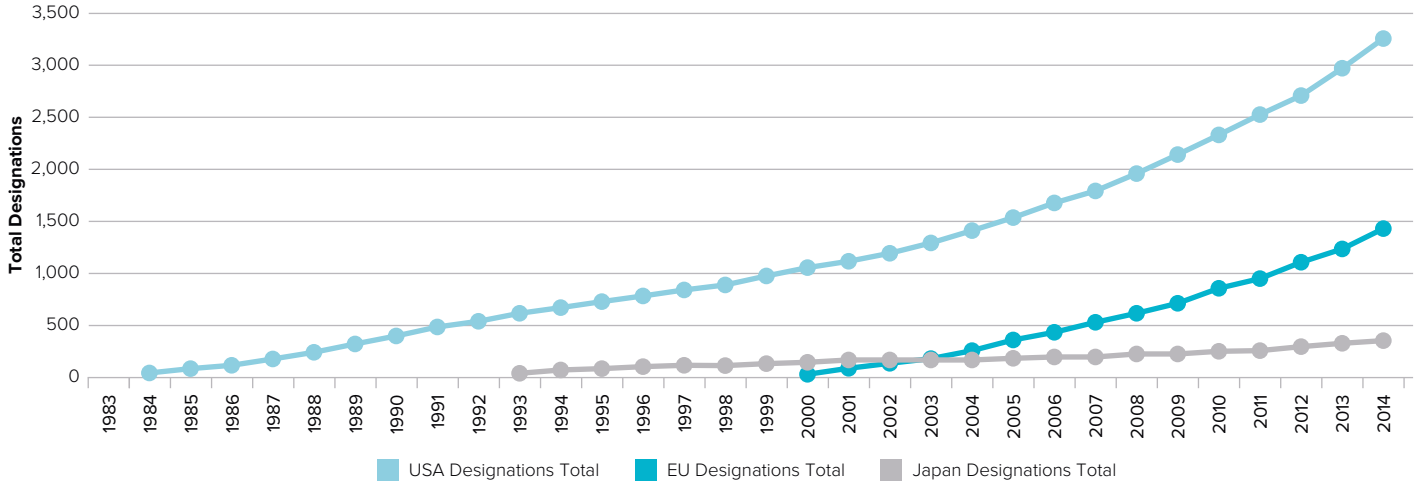
US, EU & Japan Orphan Designations per Year (2000-2014)

Source: EvaluatePharma® 30 September 2015; USA OD applications per year from PAREXEL Consulting

	Orphan Designations														
Year	2000	2001	2002	2003	2004	2005	2006	2007	2008	2009	2010	2011	2012	2013	2014
USA OD Applications	88	129	115	167	174	174	191	181	185	250	323	306	264	346	467
Growth per Year		+47%	-11%	+45%	+4%	+0%	+10%	-5%	+2%	+35%	+29%	-5%	-14%	+31%	+35%
USA Designations Accepted	70	78	64	96	132	123	142	119	166	165	195	202	190	260	291
Growth per Year		+11%	-18%	+50%	+38%	-7%	+15%	-16%	+39%	-1%	+18%	+4%	-6%	+37%	+12%
% Accepted		60%	56%	57%	76%	71%	74%	66%	90%	66%	60%	66%	72%	75%	62%
EU Designations per Yr	14	63	52	54	74	91	82	100	74	109	130	108	149	124	201
Growth per Year		+350%	-17%	+4%	+37%	+23%	-10%	+22%	-26%	+47%	+19%	-17%	+38%	-17%	+62%
Japan Designations per Yr	12	7	5	7	8	5	14	10	16	7	10	24	36	30	32
Growth per Year		-42%	-29%	+40%	+14%	-38%	+180%	-29%	+60%	-56%	+43%	+140%	+50%	-17%	+7%

USA, EU & Japan Designations Cumulative Total

Source: EvaluatePharma® 30 September 2015



Cumulative US, EU & Japan Orphan Designations & US Approvals per Year (2000-2014)

Source: EvaluatePharma® 30 September 2015

	Orphan Designations Cumulative Total														
Year	2000	2001	2002	2003	2004	2005	2006	2007	2008	2009	2010	2011	2012	2013	2014
USA Designations Total	1,057	1,135	1,199	1,295	1,427	1,550	1,692	1,811	1,977	2,142	2,337	2,539	2,729	2,989	3,280
Growth per Year		+7%	+6%	+8%	+10%	+9%	+9%	+7%	+9%	+8%	+9%	+9%	+7%	+10%	+10%
EU Designations Total	14	77	129	183	257	348	430	530	604	713	843	951	1,100	1,224	1,425
Growth per Year			+68%	+42%	+40%	+35%	+24%	+23%	+14%	+18%	+18%	+13%	+16%	+11%	+16%
Japan Designations Total	148	155	160	167	175	180	194	204	220	227	237	261	297	327	359
Growth per Year		+5%	+3%	+4%	+5%	+3%	+8%	+5%	+8%	+3%	+4%	+10%	+14%	+10%	+10%

FDA Approvals of Designations & New Drugs (NMEs/ BLAs): 2000 to 2014

FDA approves 19 new orphan drugs in 2014 vs. 16 in 2013; Orphans 38% of total FDA new drug approvals in 2014.

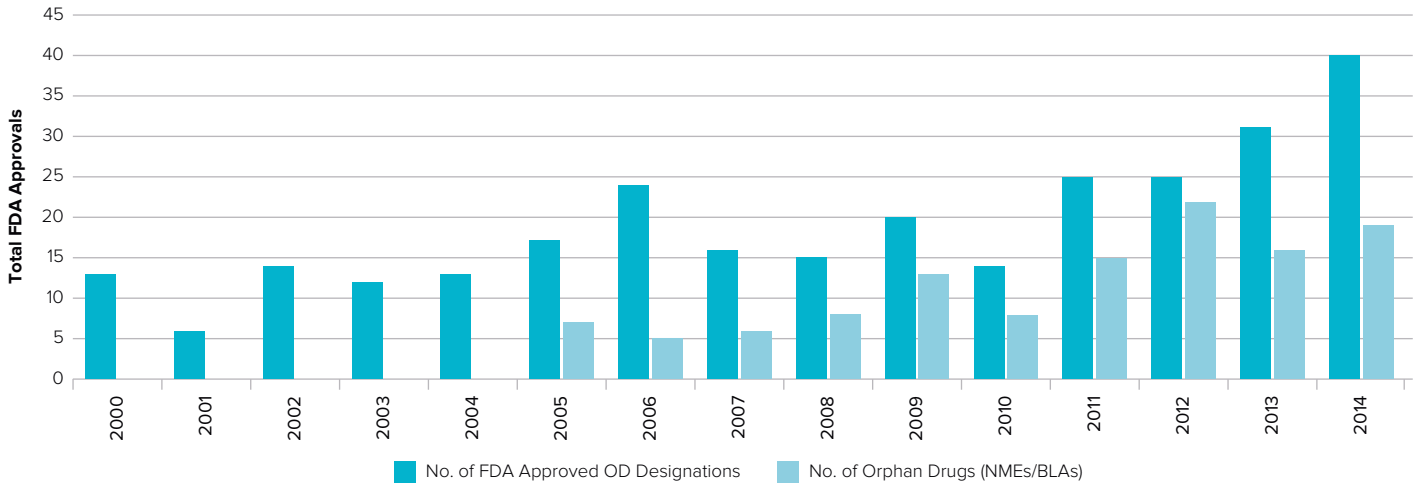
FDA approves 40 designations in 2014 vs. 31 in 2013.

EvaluatePharma® finds that 19 new orphan drugs (NMEs/ BLAs and biologicals) were approved in 2014 out of 50 new drugs. Orphan drugs represented 38% of the industry's new drug output in 2014.

Overall the FDA approved 40 designations in 2014 vs. 2013. This includes new indication approvals of already marketed products.

FDA Orphan Drug Marketing Approvals & Orphan Drugs New Drugs (NMEs/BLAs)

Source: EvaluatePharma® 30 September 2015



FDA Approved New Orphan Drug Indications and New Orphan Drugs

Source: EvaluatePharma® 30 September 2015

Year	2000	2001	2002	2003	2004	2005	2006	2007	2008	2009	2010	2011	2012	2013	2014
No. of Orphan Drugs (NMEs/BLAs)						7	5	6	8	13	8	15	22	16	19
% Chg.							-29%	+20%	+33%	+63%	-38%	+88%	+47%	-27%	+19%
NME - Orphans						6	3	5	6	9	5	11	20	15	15
BLA - Orphans						1	2	1	2	4	3	4	2	1	4
Biologicals - Orphans						1	2	2	2	6	1	3	1	3	11
FDA Approved New Orphan Drug Indications and New Orphan Drugs	13	6	14	12	13	17	24	16	15	20	14	25	25	31	40
% Chg.		-54%	+133%	-14%	+8%	+31%	+41%	-33%	-6%	+33%	-30%	+79%	+0%	+24%	+29%

Total FDA Approval Count (Orphan & Non-Orphan)

Source: EvaluatePharma® 30 September 2015

Year	2000	2001	2002	2003	2004	2005	2006	2007	2008	2009	2010	2011	2012	2013	2014
No. of NMEs Approved	27	24	17	21	31	18	18	16	21	19	15	24	33	25	30
No. of Biologicals Approved	6	8	9	14	7	10	11	10	10	15	11	11	10	10	20
Total NMEs + Biologicals	33	32	26	35	38	28	29	26	31	34	26	35	43	35	50
% Chg.		-3%	-19%	+35%	+9%	-26%	+4%	-10%	+19%	+10%	-24%	+35%	+23%	-19%	+43%
No. of Orphan Drugs (NMEs/BLAs)						7	5	6	8	13	8	15	22	16	19
as a % of Total						25%	17%	23%	26%	38%	31%	43%	51%	46%	38%

Note: Audited orphan drug NME count currently available from 2005+.

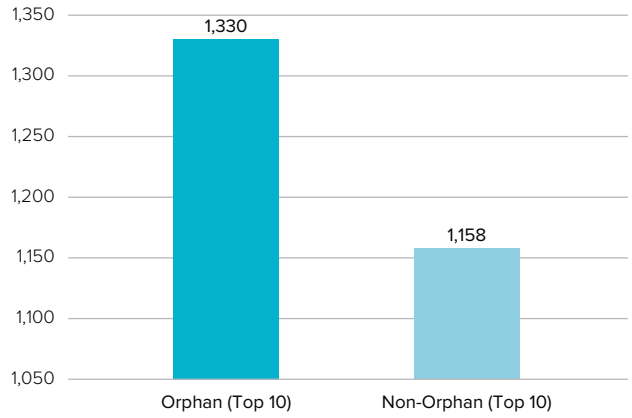
FDA New Drug Approval Analysis (NMEs/ BLAs) 2014: Orphan vs. Non-Orphan

Opdivo most promising new orphan drug approved by FDA in 2014: Top 10 orphan drugs have 155% the sales potential of non-orphan and delivered at 64% of the Phase III trial cost.

EvaluatePharma® finds that Opdivo, from Bristol-Myers Squibb , for melanoma is the most promising new orphan drug approved in 2014, with expected US sales in 2019 of $4,601m. The top 10 orphan drugs approved in 2014 are expected to sell on average $1,330m in the USA market, five years post launch. This compares with $1,158m for non-orphans approved in 2014.

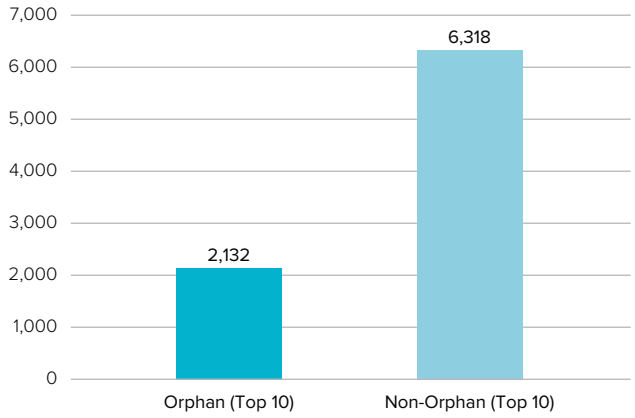
The average Phase III trial size for the top 10 orphans was 2,132 patients vs 6,318 for top 10 non-orphans approved in 2014.

Avg. USA Sales 5 Years After Launch (2014 FDA Approvals)



Avg. Phase III Trial Size (2014 FDA Approvals)

Source: EvaluatePharma® 30 September 2015



Top 10 Orphan New Molecular Entities approved in 2014: Ranked on USA Consensus Sales in 2019

Source: EvaluatePharma® 30 September 2015

Rank	Product	OD Indication	Company	Approved	US Sales ($m) 2014	US Sales ($m) 2019	Phase III Trial Size	R&D PIII Cost ($m)
1.	**Opdivo**	Melanoma	Bristol-Myers Squibb	Dec 2014	1	4,601	3,828	193
2.	**Keytruda**	Melanoma	Merck & Co	Sep 2014	48	2,798	645	102
3.	**Esbriet**	Pulmonary fibrosis, idiopathic	Roche	Oct 2014	5	1,823	4,334	218
4.	**Ofev**	Pulmonary fibrosis, idiopathic	Boehringer Ingelheim	Oct 2014	5	1,085	4,226	213
5.	**Cyramza**	Stomach cancer	Eli Lilly	Apr 2014	76	892	2,374	239
6.	**Eloctate**	Haemophilia A	Biogen	Jun 2014	58	692	394	114
7.	**Sylvant**	Castleman disease	Johnson & Johnson	Apr 2014	9	477	80	35
8.	**Zydelig**	Non-Hodgkin lymphoma (NHL); Leukaemia, chronic lymphocytic (CLL)	Gilead Sciences	Jul 2014	23	373	2,243	226
9.	**Alprolix**	Haemophilia B	Biogen	Mar 2014	72	308	251	72
10.	**Lynparza**	Ovarian cancer	AstraZeneca	Dec 2014	-	250	2,940	296
	Top 10				297	13,299	21,315	1,709
	Other				61	719		
	Total				**359**	**14,018**		
				Average for Top 10:		1,330	2,132	
				As a % of non-orphans:		115%	34%	64%

Top 10 Non-Orphan New Molecular Entities approved in 2014: Ranked on USA Consensus Sales in 2019

Source: EvaluatePharma® 30 September 2015

Rank	Product	Company	Approved	US Sales ($m) 2014	US Sales ($m) 2019	Phase III Trial Size	R&D PIII Cost ($m)
1.	**Harvoni**	Gilead Sciences	Oct 2014	2,001	3,650	2,258	228
2.	**Entyvio**	Takeda	May 2014	184	1,289	4,627	233
3.	**Viekira Pak**	AbbVie	Dec 2014	48	1,192	2,622	264
4.	**Otezla**	Celgene	Mar 2014	70	1,148	2,026	204
5.	**Jardiance**	Boehringer Ingelheim	Aug 2014	20	944	19,467	561
6.	**Plegridy**	Biogen	Aug 2014	28	812	3,336	168
7.	**Trulicity**	Eli Lilly	Sep 2014	10	744	16,090	464
8.	**Farxiga**	AstraZeneca	Jan 2014	113	709	10,049	289
9.	**Jublia**	Valeant Pharmaceuticals International	Jun 2014	69	677	1,650	166
10.	**Zerbaxa**	Merck & Co	Dec 2014	-	417	1,052	106
	Top 10			2,543	11,583	63,177	2,684
	Other			98	2,569		
	Total			**2,640**	**14,152**		
			Average for Top 10:		1,158	6,318	

Note: USA forecast sales 5 years after launch (2019) were used to rank new molecular entities.

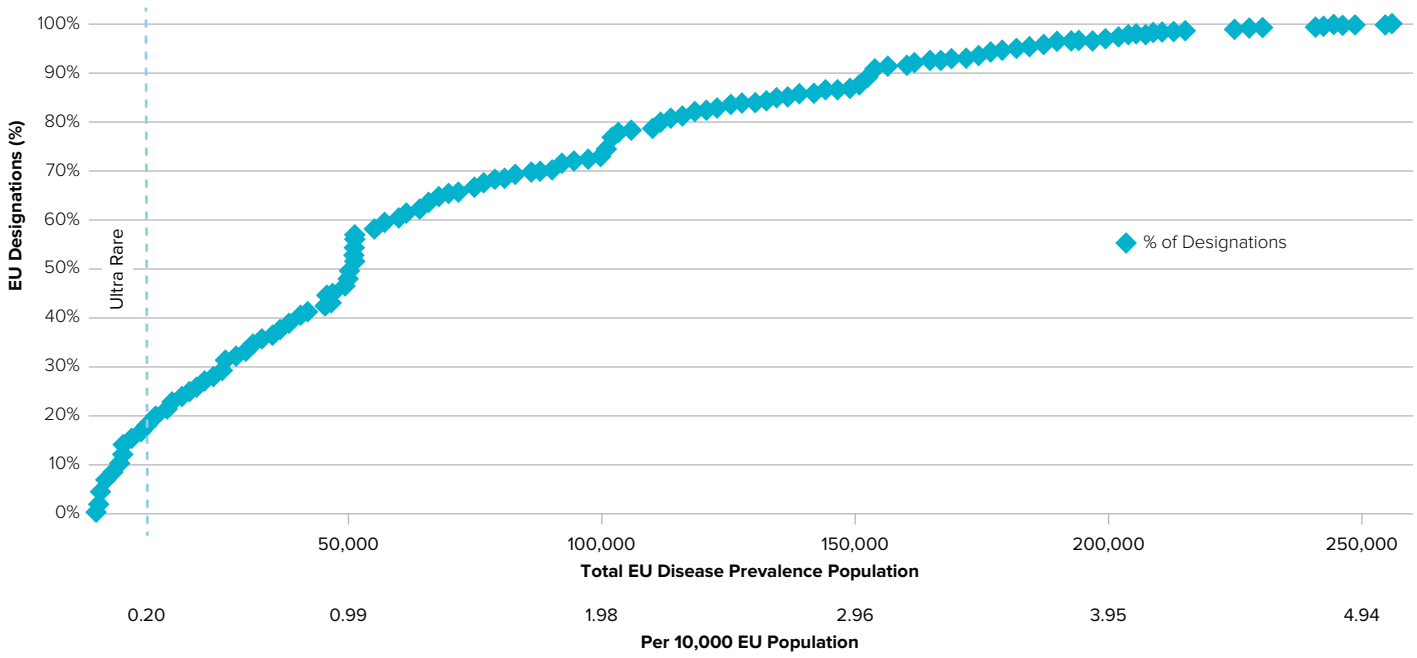
EU Orphan Designations: Historic Distribution by Prevalence

19.4% of EU orphan designations for ultra rare diseases.

EvaluatePharma® found that 19.4% (296 out of 1,525) of EU Orphan drug designations are for an ultra rare orphan disease. An ultra rare designation is classed as having an EU prevalence of 10,000 or less individuals (0.20 per 10,000). In the ultra rare segment 21.3% (63) orphan drug designations were granted for designations with a prevalence of less than 501 individuals. At the other end of the prevalence scale just 0.9% (13) of designations were granted for diseases with a prevalence of 240,000 or more individuals.

Historic Distribution of Orphan Drug Designations in the EU

Source: EvaluatePharma® 30 September 2015



Distribution and Count of EU Orphan Designations Based on Prevalence

Source: EvaluatePharma® 30 September 2015

EU Prevalence	% of Total	Cumulative % of Total	Designation Count	Designation Count 2014*	Increase in Designations	% Increase in Designations
0 - 10,000	19.4%	-	296	243	53	22%
10,000 - 20,000	7.1%	26.6%	109	102	7	7%
20,000 - 30,000	7.8%	34.4%	119	103	16	16%
30,000 - 40,000	5.6%	40.0%	86	74	12	16%
40,000 - 50,000	10.1%	50.1%	154	145	9	6%
50,000 - 100,000	23.3%	73.4%	355	301	54	18%
100,000 - 150,000	13.6%	87.0%	207	172	35	20%
150,000 - 200,000	10.0%	96.9%	152	124	28	23%
200,000 - 260,000	3.1%	100.0%	47	38	9	24%
Total	**100.0%**		**1525**	**1302**	**223**	

Note: Based on available EU prevalence data for a specific designation. EU total population taken to be 506,300,000. EvaluatePharma® defined an ultra rare orphan drug as a disease affecting fewer than 20 individuals per million of population (0.2 per 10,000).

* Count based on designation counts from EvaluatePharma® Orphan Drug report 2014.

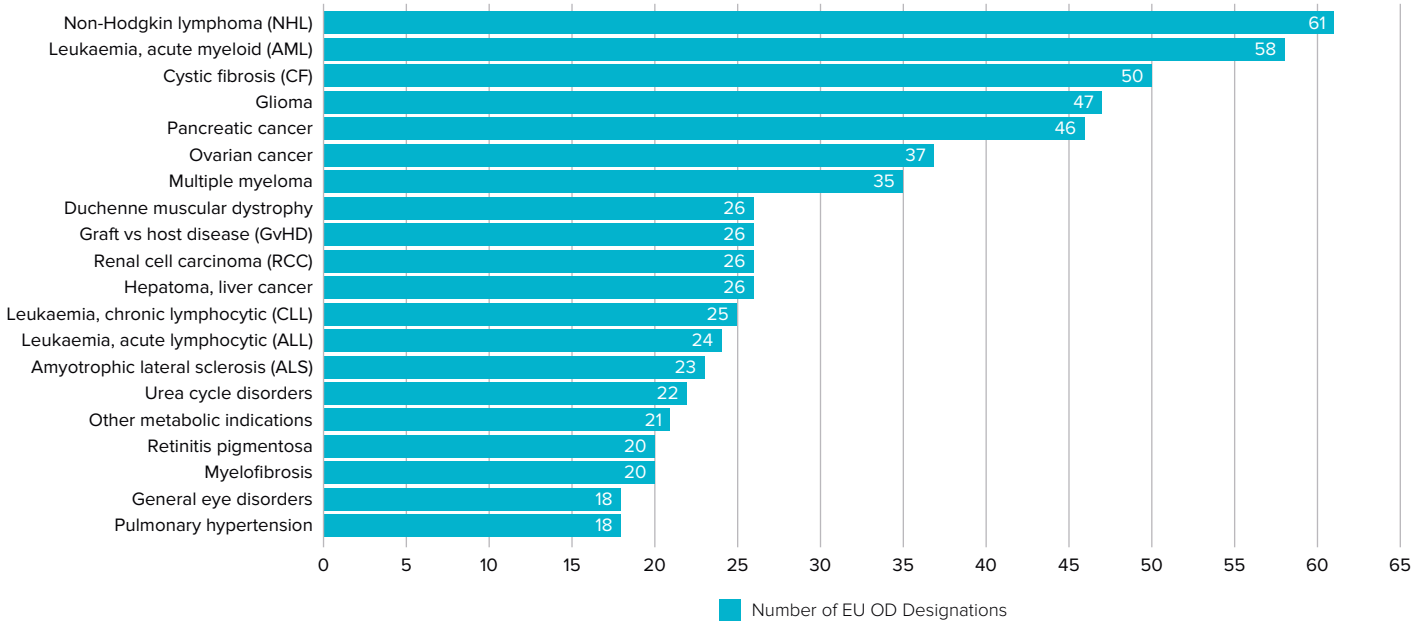
EU Orphan Designations: Top 20 Indications

Non-Hodgkin's lymphoma (NHL) is the indication with most filed orphan drug designations in EU.

EvaluatePharma® finds that non-Hodgkin's lymphoma (NHL) is the indication with the most orphan designations in the EU. Half (10) of the top 20 indications fall within EvaluatePharma's® Cancer categorisation with 5 in the Blood & Blood Forming Malignancies subcategory. Other notable indications include historically well defined populations and indications such as cystic fibrosis and Duchenne muscular dystrophy. The Top 20 indications account for 41% of indications sought in the EU.

Top 20 EU Orphan Designations

Source: EvaluatePharma® 30 September 2015



Top 20 Orphan Drug Designation Indications in the EU

Source: EvaluatePharma® 30 September 2015

Rank	Indication	Total EU Designations	As a %	EU Prevalence (per 10,000)
1.	Non-Hodgkin lymphoma (NHL)	61	4%	3.6
2.	Leukaemia, acute myeloid (AML)	58	4%	2.7
3.	Cystic fibrosis (CF)	50	3%	1.3
4.	Glioma	47	3%	2.2
5.	Pancreatic cancer	46	3%	2.0
6.	Ovarian cancer	37	2%	3.5
7.	Multiple myeloma	35	2%	3.2
8.	Duchenne muscular dystrophy	26	2%	0.5
9.	Graft vs host disease (GvHD)	26	2%	1.0
10.	Renal cell carcinoma (RCC)	26	2%	4.2

Top 11-20 continued over...

EU Orphan Designations: Top 20 Indications

Rank	Indication	Total EU Designations	As a %	EU Prevalence (per 10,000)
11.	Hepatoma, liver cancer	26	2%	2.7
12.	Leukaemia, chronic lymphocytic (CLL)	25	2%	4.0
13.	Leukaemia, acute lymphocytic (ALL)	24	2%	1.2
14.	Amyotrophic lateral sclerosis (ALS)	23	1%	1.0
15.	Urea cycle disorders	22	1%	1.2
16.	Other metabolic indications	21	1%	0.4
17.	Retinitis pigmentosa	20	1%	3.7
18.	Myelofibrosis	20	1%	1.0
19.	General eye disorders	18	1%	2.3
20.	Pulmonary hypertension	18	1%	2.2
	Sub Total	**629**	**41%**	
	Other	916	59%	
	Total	**1545**	**100%**	

Note: Designations assigned based on closest possible match to existing indications within EvaluatePharma®. For example: EU Designation: Adult acute myeloid leukaemia with 11q23 (MLL) abnormalities is mapped to Leukaemia, acute myeloid (AML).

Evaluate®

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www.evaluate.com

Evaluate Headquarters
Evaluate Ltd.
11-29 Fashion Street
London E1 6PX
United Kingdom
T +44 (0)20 7377 0800
F +44 (0)20 7539 1801

Evaluate North America
EvaluatePharma USA Inc.
15 Broad Street, Suite 401
Boston, MA 02109, USA
T +1 617 573 9450
F +1 617 573 9542

Evaluate Japan
Evaluate Japan KK
Akasaka Garden City 4F
4-15-1 Akasaka, Minato-ku
Tokyo 107-0052, Japan
T +81 (0)80 1164 4754