Table 1. Voucher Obtainment Process and Benefit and Criteria for Use

Voucher Obtainment			Voucher Usage	
Voucher Type	Application Requirement	Eligibility Requirement	Potential Benefit	Criteria for Use
Rare Pediatric Disease Voucher[1]	• Sponsor must submit an application for a drug or biologic intended to prevent a rare pediatric disease • The application must be designated as a rare pediatric disease application • Sponsor may choose to request a rare pediatric disease designation for the drug or biologic prior to submission, but it is not required	• The drug or biologic may not contain any active ingredient previously approved in any drug application. The rare pediatric disease application must be eligible for a priority review*	• Allows priority review for a drug or biologic product that would not otherwise qualify • May be transferred or sold to another sponsor • Allowed unlimited transfers of voucher	• Sponsor must notify FDA of intent to use voucher at least 90 days prior to submitting an application 1,2,3 • Payment of FY 2017 voucher usage fee of $2,706,000.00 upon submission

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Table 1. Voucher Obtainment Process and Benefit and Criteria for Use, Continued

Voucher Obtainment			Voucher Usage	
Voucher Type	Application Requirement	Eligibility Requirement	Potential Benefit	Criteria for Use
Neglected Tropical Disease Voucher[2,3]	• Submission of an application for a drug or biologic intended to prevent or treat a listed tropical disease	• The drug or biologic may not contain any active ingredient previously approved in any drug application • The tropical disease voucher application must qualify for a priority review*	• Allows priority review for a drug or biologic product that would not otherwise qualify • May be transferred or sold to another sponsor • Allowed unlimited transfers of voucher[2,3]	• Sponsor must notify FDA of intent to use voucher at least 90 days prior to submitting an application[1,2,3] • Payment of FY 2017 voucher usage fee of $2,706,000.00 upon submission

1 *Priority review application are completed within 6-months verses a standard 10-month review
2 **U.S. FDA. Draft Guidance for Industry: Rare Pediatric Disease Priority Review Vouchers. November 2014. Available at: http://www.fda.gov/downloads/RegulatoryInformation/Guidances/UCM423325.pdf**
3 **U.S. FDA. Draft Guidance For Industry: Tropical Disease Priority Review Vouchers. October 2016. Available at: http://www.fda.gov/downloads/Drugs/Guidances/UCM080599.pdf**
4 Senate. Senate Bill 2917; Adding Ebola to the FDA Priority Review Voucher Program Act. December 2014. Available at**: https://www.congress.gov/113/bills/s2917/BILLS-113s2917enr.pdf**

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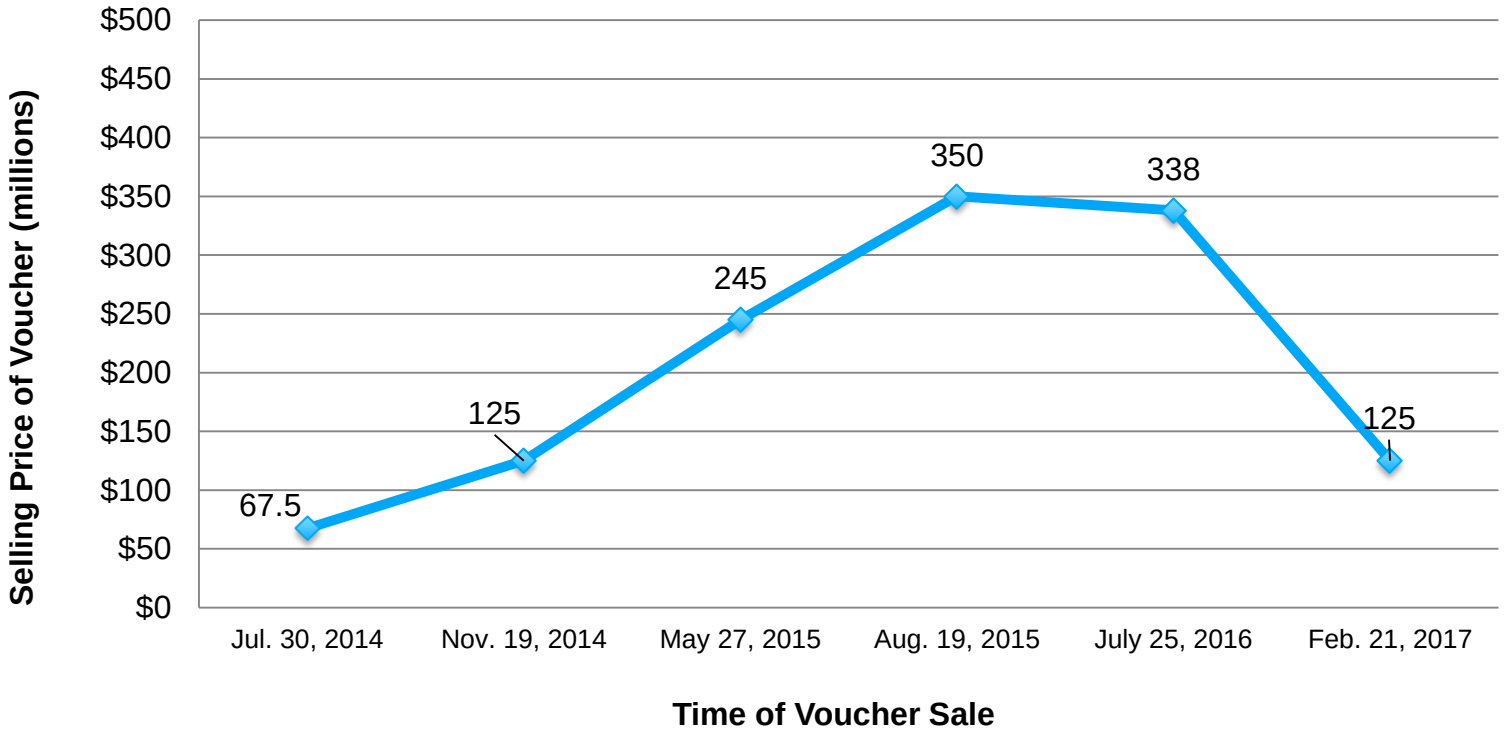
Figure 1. Priority Review Voucher Sales Over Time*



*PRVs sales within chart include both Rare Pediatric and Neglected Tropical Disease Voucher Sales

** U.S. Food and Drug Administration (FDA), Office of Orphan Products Development. Narrative by Activity. Available at: https://www.fda.gov/downloads/AboutFDA/ReportsManualsForms/Reports/BudgetReports/UCM488554.pdf(accessed 2/23/2017)

§Gilead announced in Q2 earnings of a voucher purchase in context of its R&D expenses. Seller and price is undisclosed but their R&D expense increase (of $338m) is credited to PRV purchase and progression of clinical studies. As a result the price may be ≤$338m.

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Table 2. Snapshot of Neglected Tropical Disease Priority Review Vouchers Issued

Neglected Tropical Disease				
Date	**Company**	**Drug**	**Indication**	**Sold/Unsold**
Apr. 2009	Novartis	Coartem®	Malaria	Unsold (used)
Dec. 2012	Janssen	Sirturo®	Tuberculosis	Undisclosed
Mar. 2014	Knight Therapeutics	Impavido®	Leishmaniasis	Sold
June 2016	PaxVax Bermuda	Vaxchora®	Cholera	Undisclosed

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Table 3. Snapshot of Rare Pediatric Disease Priority Review Vouchers Issued

Rare Pediatric Disease

Date	Company	Drug	Indication	Sold/Unsold
Feb. 2014	BioMarin	Vimizim®	Morquio A syndrome	Sold
Mar. 2015	United Therapeutics	Unituxin®	High-risk neuroblastoma	Sold
Mar. 2015	Asklepion Pharmaceuticals (acquired by Retrophin)	Cholbam®	Rare bile acid synthesis disorders	Sold
Sep. 2015	Wellstat Therapeutics	Xuriden®	Hereditary orotic aciduria	Sold
Oct. 2015	Alexion Pharmaceuticals	Strensiq®	Hypophosphatasia	Undisclosed
Dec. 2015	Alexion Pharmaceuticals	Kanuma®	Lysosomal acid lipase deficiency	Undisclosed
Sep. 2016	Sarepta Therapeutics	Exondys 51®	Duchenne muscular dystrophy	Sold
Dec. 2016	Ionis Pharmaceuticals (acquired by Biogen)	Spinraza®	Spinal muscular atrophy	Unsold
Feb. 2017	Marathon Pharmaceuticals	Emflaza®	Duchenne muscular dystrophy	Unsold

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Figure 2. The Number of Vouchers Awarded by PRV program category

	Number of Vouchers	Drug Names
Neglected Tropical Disease Priority Review Vouchers	4	Coartem®, Sirturo® , Impavido® , Vaxchora®
Rare Pediatric Disease Priority Review Vouchers	9	Vimizim®, Unituxin®, Cholbam®, Xuriden®, Strensiq®, Kanuma®, Exondys 51®, Spiranza®, Emflaza®,

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