Start New Drug Filing * indicates a required field		
NDC*	Trade Name*	Chemical Name*
71274-0170-60	Joenja	Leniolisib

Marketing Description*	Marketing Description Trade Secret Indicator*
Joenja (leniolisib) was just approved by the FDA at the end of Q1 2023. Joenja is the first FDA approved treatment for APDS. APDS or activated PI3K delta syndrome (previously known as PASLI Disease) is a rare and progressive primary immunodeficiency that was first characterized in 2013. Joenja is designed to correct the underlying immune defect in APDS patients, helping the immune system function normally. Pharming is planning standard promotional efforts that will focus on disease and branded education through a limited specialty sales force and printed marketing materials focused on immunology and other specialists treating PI disorders. This also includes digital efforts via product websites for both patients and physicians. Pharming offers a starter program to help patients start on therapy. Pharming will participate in scientific meetings attended by healthcare professionals. Pharming reported global marketing and sales (M&S) expenses of $85.8M for 2022. Marketing expenses for Joenja are included in the global M&S expenses along with many other commercial and non-commercial operating expenses.	N

Marketing description: Include the dollar amount spent to market this drug and describe the marketing used in the introduction of the new prescription drug, including: • Direct-to-consumer media advertisements on platforms, such as TV, magazines, radio, social media, blogs, billboards, mobile applications, or other web-based media. • Direct-to-consumer promotional incentives, such as free trial offers, rebates, coupons, and other utilization incentives if different from the patient assistance program. • Promotion of the drug to physicians or other health professionals, such as professional detailing, free drug samples, sponsorships for continuing education for health professionals, gifts, conference events, seminars, or other promotional activities. • Other paid advertising or promotion to consumers or health care providers. Compliance tip: Marketing description must include information about the company's spending on marketing and the dollar amounts the company spent. For a generic drug, if there was no marketing for the drug, report there was no marketing. If the company marketed the generic drug, report the amount spent on marketing.

Marketing Description Trade Secret Justification*	Wholesale Acquisition Cost (WAC)*
Note: only populate if marking previous field as a Trade Secret	45000

Wholesale Acquisition Cost (WAC) Trade Secret Indicator*	Wholesale Acquisition Cost (WAC) Trade Secret Justification*	Dosage*
N	Note: only populate if marking previous field as a Trade Secret	Twice Daily

Dosage Trade Secret Indicator*	Dosage Trade Secret Justification*
N	Note: only populate if marking previous field as a Trade Secret

Pricing Methodology*

Joenja (leniolisib) received FDA approval on March 24, 2023 and becomes the first and only FDA approved treatment for APDS in adult and pediatric patients 12 years of age and older. Joenja is a precision medicine, targeting the PI3K delta pathway and designed to treat the root cause of APDS treating both immune deficiency and dysregulation. APDS is a rare, primary immunodeficiency (PI) disorder, first characterized in 2013 with an estimated prevalence of 1-2 per million worldwide. A definitive diagnosis of APDS can be determined by genetic test confirmation of a variant in one of the genes associated with the disorder *(PIK3CD or PIK3R1).* Hyperactive PI3Kδ results in dysregulated B and T cell development leading to a diverse set of progressive clinical manifestations such as severe and recurrent sinopulmonary infections, lymphoproliferation, enteropathy, autoimmunity, bronchiectasis, and malignancies such as lymphoma. Pharming conducted extensive market research and analysis of current market pricing for comparable ultra-orphan drugs in determining the price of Joenja which included broad-based physician and payor feedback. Qualitative and quantitative

pricing research was conducted including assessment of the prevalence to price ratio established for recently approved ultra-orphan drugs, severity of disorder, assessment of overall unmet medical need in a highly heterogeneous, ultra-orphan indication, clinicians

perceived value of the treatment inclusive of safety and efficacy data, and expectations for usage within the patient population. Additional financial and non-financial factors were utilized in establishing the list price which included:

· Costs associated with the investment in research and development of leniolisib which included completion of the clinical trial for FDA submission.

· Investment in the funding of supplemental pediatric clinical trials to establish future access to patients under the age of 12.

· Funding the commercial infrastructure to launch and sustain patient support services to provide access to

Pricing methodology: Describe the methodology used to establish the price (WAC) of the new prescription drug, including a narrative description and explanation of all major financial and nonfinancial factors that influenced the decision to set the price of the drug. Include an explanation of any cost-based pricing, quality-adjusted pricing, value□based pricing, or other models and strategies used to establish the price. Include the description of any fixed costs, variable costs, quality measures, utilization estimates, and other relevant information. For generic drugs, specify in detail what percentages were applied in pricing this particular drug or confirm that no percentage difference or comparison to brand name or other generic equivalents was used in determining the price. Compliance tip: Report clear and specific information. If the company did not use any drug comparisons for determining the price, clearly confirm in writing th

	Filing Det *** indicates a req**
Pricing Methodology Trade Secret Indicator*	Pricing Methodology Trade Secret Justification*
N	Note: only populate if marking previous field as a Trade Secret

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FDA Priority or Breakthrough (select from dropdown list)*	Acquisition Cost (required if the drug was acquired)
No	$20M

Acquisition Cost Trade Secret Indicator*	Acquisition Cost Trade Secret Justification*
N	Note: only populate if marking previous field as a Trade Secret

Acquisition Date (required if the drug was acquired)	Acquisition Date Trade Secret Indicator*
8/13/2019	N

Acquisition Date Trade Secret Justification*	Estimated Avg. Number of Patients per Month*
Note: only populate if marking previous field as a Trade Secret	5-10 new patients per month in the US. Estimated to be 7 patients per month.

Estimated average number of patients per month: Estimate of the average number of patients who will be prescribed the new prescription drug each month in the U.S. Include a description of any epidemiologic studies or analyses on incidence and prevalence of the conditions the drug targets and other relevant information used to estimate the average number of patients. Compliance tip: Brand-name drugs are expected to have an estimate of the number of patients per month; “0” and “1” are not acceptable for reporting this information. To provide more information regarding how the company arrived at the estimate, use the next data field (“Patients per Month Description”) to report the information.

Estimated Avg. Number of Patients per Month Trade Secret Indicator*	Estimated Avg. Number of Patients per Month Trade Secret Justification*
N	Note: only populate if marking previous field as a Trade Secret

Patients per Month Description	Patients per Month Description Trade Secret Indicator*
APDS was only first characterized in 2013 and data on prevalence is limited. However, the prevalence is estimated to be 1-2 patients per million. According to the United States Census Bureau, the estimated population of Oregan is approximately 4.2 million suggesting that there could be approximately 4 patients with ADPS in the state of Oregan. According to published literature, patients with APDS often experience a 7-year diagnostic day. Of note, it is believed that approximately 25% of APDS patients are below the age of 12 and therefore would not meet the FDA-approved indication which states Joenja is for APDS patients 12 years of age and older.	N

Patients per Month Description Trade Secret Justification*	Research and development costs using International Public Funds*
Note: only populate if marking previous field as a Trade Secret	0

Research and development costs using International Public Funds Trade Secret Indicator*	Research and development costs using International Public Funds Trade Secret Justification*
N	Note: only populate if marking previous field as a Trade Secret

Research and development costs using USA Federal Public Funds	Research and development costs using USA Federal Public Funds Trade Secret Indicator*
0	N

Research and development costs using USA Federal Public Funds Trade Secret Justification*	Research and development costs using All States Public Funds
Note: only populate if marking previous field as a Trade Secret	0

Research and Development Costs Using * indicates a required field	
Research and development costs using All States Public Funds Trade Secret Indicator*	Research and development costs using All States Public Funds Trade Secret Justification*
N	Note: only populate if marking previous field as a Trade Secret

Research and development costs using All Local Public Funds	Research and development costs using All Local Public Funds Trade Secret Indicator*
0	N

Research and development costs using All Local Public Funds Trade Secret Justification*	Research and development costs using Public Funds Sources and Uses
Note: only populate if marking previous field as a Trade Secret	0

Research and development costs using Public Funds Sources and Uses Trade Secret Indicator*	Research and development costs using Public Funds Sources and Uses Trade Secret Justification*
N	Note: only populate if marking previous field as a Trade Secret

Documents (only complete if uploading a separate d * indicate	
Document Type (select from dropdown list)*	Reason for Inclusion*

Average Patients per Month

Drug Price, Dosage or Course of Treatment
FDA Priority of Breakthrough Designation

Marketing Description
Other Supporting Documentation
Pricing Methodology
Public Funds

document attachment in addition to reported information) s a required field	
Document Upload (select file to be uploaded with report)*	Document Upload Trade Secret Indicator*
	Y/N

Document Upload Trade Secret Justification*

Note: only populate if marking previous field as a Trade Secret