

**PAYING FOR CURES**

**Ask PAM: "How many?"**

Individual Indication Workbook in action

**How many treatment-eligible people are in my population?**



**Get the Individual Indication Workbook**



**Visit Paying for Cures**

The Pipeline Analysis and Modeling (PAM) in the NEWDIGS Paying for Cures Toolkit maintains a unique, detailed, indication-by-indication analysis to estimate the expected volume of durable cell and gene therapies (CGT) likely to be available on the US market in the coming years. This indication specific analysis is available to you in the Individual Indication Workbook. You can use this workbook to calculate the likelihood and scale of treatment-eligible patients in your population.

**Use case examples**

A commercial payer covering a new gene therapy wants to understand the potential impact of adverse selection in their service area.

A hospital wants to understand the resource need within their service area for a new gene therapy.

A self-insured employer wants to know the likelihood of paying for a new cell or gene therapy given the size of their company.

A developer wants to discuss their product with a payer and how many patients they may anticipate needing the product.

PAM provides disease specific incidence and prevalence for the selected disease.

**Select an indication**

*Step 1: Please select the relevant indication in the green pull-down menus*  
Based on your selections the team's estimate of total clinically-relevant US incidence and prevalence will populate.

Indication Menus -->	Therapeutic class	Disease	Sub population	Total US incidence estimate	Total US prevalence estimate
	Hematology	Sickle cell disease	All	1,650	85,000

**DEFINITIONS:** Indicating a sub population of "All" results in the total population with the indicated disease/condition.  
Indication a sub population other than "All" will provide estimates of incidence and prevalence for the clinically relevant population.  
**Clinically relevant population**

US Population - 2020 census data	331,500,000	Total US incidence per 100,000	0.5	Total US prevalence per 100,000	25.6
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The clinically relevant population for CGT is provided when the therapy targeted gene is selected - for example, BCL11A for Sickle Cell disease treated with Casgevy.

Indication Menus -->	Therapeutic class	Disease	Sub population	Total US incidence estimate	Total US prevalence estimate
	Hematology	Sickle cell disease	BCL11A	770	13,300

**DEFINITIONS:** Indicating a sub population of "All" results in the total population with the indicated disease/condition.  
Indication a sub population other than "All" will provide estimates of incidence and prevalence for the clinically relevant population.  
**Clinically relevant population**

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**Individual Indication Worksheet in action: Ask PAM, “How many?”**

The clinically relevant sickle cell population by payer type is provided when selecting a payer type option.

**Step 2: Customize the tool to reflect payer type**

Select diseases/conditions are more commonly associated with lives covered under Medicare or Medicaid. This section makes adjustments to reflect that modification of incidence and prevalence within the US population.

Select payer type from green pull-down menu below

Plan specific incidence and prevalence Medicaid

"Medicaid" incidence estimate	"Medicaid" prevalence estimate
490	6,300

PAM has identified the impact of a specific sickle cell gene therapy by payer and has found the prevalent number of treatment eligible patients to be slightly higher for all other payers that are not Medicaid or Medicare, primarily associated with eligibility age criteria for Medicaid.

"Medicare" incidence estimate	"Medicare" prevalence estimate	"Not Medicare or Medicaid" incidence estimate	"Not Medicare or Medicaid" prevalence estimate
0	210	280	6,790

Population size adjustments can tailor PAM to more specific questions of “How many?”

**Step 3: Ratio the "Not Medicare or Medicaid" population to your plan**

Your Plan Size (# of members) 130,000

Plan-specific incidence	Plan-specific prevalence
0.2	4.5

Note: It is recommended to indicate lives within the payer type segment selected in step 2. "General Population" payer type should be used if analyzing multiple lines of business concurrently.



**About Paying for Cures**

Your toolkit to understand CGT financing and to lead change in your organization

Since 2019, NEWDIGS has provided cutting-edge free research and tools for organizations adapting to the challenge of making innovative cell and gene therapies financially sustainable for the healthcare ecosystem and accessible to patients. At Paying for Cures, you'll find:

- Detailed descriptions of financing challenges
- Precision Financing Solutions to meet the challenges
- Views from other stakeholders to understand the system
- Research, media, tools, and presentation templates you can use to develop your own solutions

<https://newdigs.tuftsmedicalcenter.org/payingforcures/>

**RELATED ARTICLES AND RESEARCH**

Financial challenges of cell and gene therapies	Preparing for CGT financial impact	How are cell and gene therapies distinctive?	CGT product profile challenges
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