

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 for approved and pipeline therapies.

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
	Neurological	Muscular atrophy, spinal	All	425	5,300

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

US Population - 2020 census data	331,500,000	Total US incidence per 100,000	0.1	Total US prevalence per 100,000	1.6
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The clinically relevant population for CGT is provided when the therapy targeted gene is selected—for example, SMN1.

Indication Menus -->	Therapeutic class	Disease	Sub population	Total US incidence estimate	Total US prevalence estimate
	Neurological	Muscular atrophy, spinal	SMN1	250	167

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.

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

The clinically relevant spinal muscular atrophy Type 1 population by payer type is provided when selecting a payer type option.

Payer type adjustment	
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	125
"Medicaid" prevalence estimate	83

Payer may make further adjustments for the size of their population to specifically estimate “How many?” for their plan.

Population size adjustments	
Step 3: Ratio the "Medicaid" population to your plan	
Your Plan Size (# of members)	400,000
Plan-specific incidence	0.7
Plan-specific prevalence	0.5



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/>

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