PAYING FOR CURES

Ask PAM: "How likely and how soon?"

Individual Indication Workbook in action

How many treatment-eligible people are likely to treat and how long before they treat?

Get the Individual Indication Workbook

Visit Paying for Cures

The Pipeline Analysis and Modeling (PAM) in the NEWDIGS Paying for Cures Toolkit provides an indication-by-indication analysis to estimate the number of patients meeting the FDA label treatment criteria. But how many are likely to be treated? Numerous factors beyond treatment eligibility will influence a product's use. You can use the PAM workbook to learn the expectations for "likely to treatment" numbers made by NEWDIGS researchers and modify results based on your expectations.

Use case examples

A benefits consultant wants to use NEWDIGS researcher's treatment adoption expectations to advise on benefit and payment strategies for a new CGT.

A finance director wants to prepare a multi-year plan budget using various treatment adoption scenarios vis-à-vis a prevalent populations' likely treatment with a new gene therapy.

A hospital wants to anticipate potential patient volume over the next 5-10 years based on specialized gene or cell therapies.

The Market Adjustment tab of the Individual Indication workbook addresses market penetration and timing for market uptake or adoption. The workbook provides guidance default numbers for the number of patients likely to be treated. The workbook also provides Indication specific adoption variables for your consideration in defining within the orange boxes, your own modifiers to the total incident and prevalent population likely to be treated. Estimates of those likely to be treated are based on factors such as disease severity and alternative treatments. There are key questions to help you with determining the modification of those eligible to treat to those likely to be treated.

These are the default values for the % of Sickle cell disease treatment eligible patients that will be treated.

Incidence Modifier	Prevalence Modifier
80%	75%

You may override the default values by entering the % of treatment eligible patients you expect to be treated.

Incidence modifier	Prevalence modifier
80%	15%

Sickle cell disease **HBB**

Age at onset	Impact on life expectancy	Therapeutic options	Therapeutic cost (annual)	Symptoms
Genetic (HBB, BCL11A) Diagnosis:	54 years	Prophylactic penicillin up to age 5 Hydroxyurea, blood transfusion Allogeneic bone marrow transplantation Endari (L-glutamine) Adakveo (Crizanlizumab) Oxbryta (Voxelotor)	$322-$2,251, $? $892,700 $14,571-$43,714 $36,770-$147,082 (20kg-80kg) $165,240	Anemia, repeated infections, periodic episodes of pain

Questions to consider for modifying use by the treatment eligible

1. To what degree would the therapy's impact on life expectancy promote product demand?
2. To what degree would symptom severity and impact on quality of life promote product demand?
3. To what degree would alternative treatments or costs for current standard of care promote product demand?

Individual Indication Worksheet in action: Ask PAM, "How likely and how soon?"

The table below illustrates how from a prevalent population of approximately 35 patients eligible for sickle cell gene therapy is adjusted to 26 (default value) or 17 (end-user value) when modifying for the percentage of patients likely to treat in a Medicaid plan population of 300,000.

	Total Eligible pop	NEWDIGS Estimated "likely to treat"	End-user estimated "likely to treat"
Plan specific incidence	2.7	80% = 2.2	80% = 2.2
Plan specific prevalence	34.6	75% = 26	50% = 17.3

How quickly those "likely to treat" access the treatment is evaluated in the Market uptake/adoption section of the Market Assessment tab. Uptake will vary based on a variety of factors including product production, insurance coverage, provider availability, patient trust, etc. PAM defaults to 100% treatment in year 1 for both incident and prevalent populations likely to treat. However, you can make modifications by entering data in the orange cells.

Adoption curve

The model's default assumption is that 100% of eligible incident patients are treated each year and 100% of prevalent patients are treated in the first year. You may wish to modify theses assumptions. For example, you might want to assume a more gradual ramp in, with fewer than 100% of eligible patients being treated in the early years.

Do you want to use the default assumptions? No

Uptake assumptions for incident patients:	2024	2025	2026	2027	2028
	10%	40%	80%	90%	90%

Note that untreated incident patients each year are added to the prevalent pool

Uptake assumption for prevalent patients: 5 This is the number of years taken to treat all prevalent patients. (As far as possible the same number of patients

% of prevalent patient pool treated each year	2024	2025	2026	2027	2028
	20%	30%	55%	65%	80%

% prevalent patients remaining eligible: 100% Most commonly this would be 100%. A lower percentage might be used to reflect mortality in the prevalent popu

Expected treated patients given your uptake/adoption assumptions

	2024	2025	2026	2027	2028
Incident patients treated, by year (#)	0	1	2	2	2
Prevalent patients treated, by year (#)	3	5	7	3	2
Total patients treated (#)	**3**	**6**	**9**	**5**	**4**

Continuing our example of a 300,000 life Medicaid plan, the treatment adoption rate for incidence patients is set in the orange boxes. The uptake assumption to treat for prevalent patients "likely to be treated" is set as the number of years to treat all prevalent patients. The table below presents the different projections of treated patients based upon prevalent patient uptake assumptions of 3 yrs., 5 yrs., and 7 yrs. (maintaining incident uptake assumptions as presented above). Once all prevalent patients are treated, the Medicaid plan may expect 2 incident patients per year. Use of the Market Assessment tab helps quantify the potential treatment impact to answer questions such as:

1. What is the financial impact of three years of patient treatments costing 6 – 9 times $X?
2. What is the financial impact of multiple years of treatment costing 5 times $X?

Prevalence speed of uptake	2024	2025	2026	2027	2028	2029	2030	2031
Total patients treated (#) - uptake 3 yrs	6	8	9	2	2	2	2	2
Total patients treated (#) - uptake 5 yrs	3	5	6	7	6	2	2	2
Total patients treated (#) - uptake 7 yrs	2	4	5	5	5	5	5	2

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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