

ICER SNAPSHOT

Reviewed by: Foundation Fighting Blindness and Prevent Blindness

These organizations are not responsible for the final contents of ICER's Report or Snapshot, nor should their review be assumed to support any part of ICER's findings.

The ICER Snapshot is a summary designed to help patients and the broader community learn about the key results and recommendations from ICER's [2025 Final Evidence Report on Sonpiretogene Isteparvovec for Retinitis Pigmentosa](#).

The information included is up to date as of May 2025. New information about this therapy may become available, but is not captured here.

What's Included in this Snapshot?

What is Retinitis Pigmentosa?

Impact on Patients and Families

Treatment: Benefits and Risks

Treatment: What's A Fair Price?

Policy Recommendations & Impact of Engagement



| | |
|---------|---|
| 85 | 1 |
| 293 | 2 |
| 8754 | 3 |
| 63952 | 4 |
| 428356 | 5 |
| 3746285 | 6 |
| 7264793 | 7 |
| 3875264 | 8 |

What is Retinitis Pigmentosa?

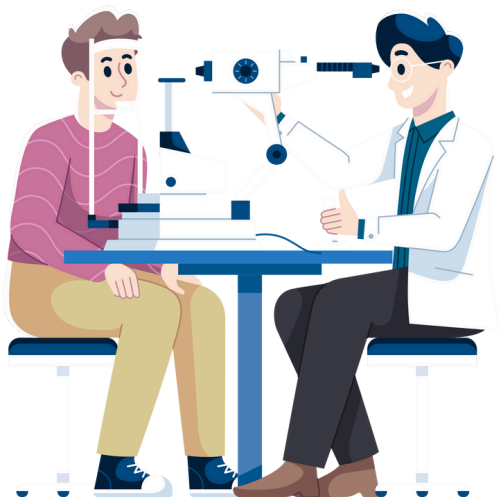


Retinitis pigmentosa (RP) is a group of **eye diseases** that affect the retina (layer of tissue in the back of the eye). RP progressively breaks down the photoreceptors (cells that convert light into visual signals) in the retina that allow you to see. Around 100,000 people are affected in the US, and over 10% of this population develop advanced RP, which only allow them to see hand movement or count fingers. Some people develop total blindness. While RP is not normally a deadly disease, the symptoms and damage caused can lead to harmful outcomes and expensive hospital bills.

Impact on Patients and Families

Access to visual aids, assistive technology, vision rehabilitation, orientation and mobility training, and home modifications are needed to adapt to vision changes and may have added out-of-pocket costs.

Due to the progressive nature of this eye disease, people with RP are often challenged by continually having to adapt and adjust to vision changes.



We heard that a loss of light perception can be tough and socially isolating, and that a slight improvement in vision may be helpful.

There are significant emotional, physical, and financial impacts on caregivers, particularly for individuals who are less able to cope and adapt to severe vision loss.

Treatment of Focus & Clinical Context

SONPIRETIGENE ISTAPARVOVEC

Sonpiretogene isteparvovec, made by Nanoscope Therapeutics, is a new gene therapy for people who have advanced RP with severe vision loss.

DOSING



This gene therapy is delivered through a one-time injection into the eye.

HOW IT WORKS

Sonpiretogene delivers light-sensitive proteins, called **opsins**, into the eye. Opsins change light into electrical signals that allow the brain to see visual images. By delivering opsins into the eye, it allows non-light sensitive cells in the eye to react to light and possibly restore vision.

Nanoscope is likely to submit sonpiretogene isteparvovec for FDA approval later this year as a treatment for patients with advanced RP.

How Did Clinical Trials Define Outcomes for RP?



Best Corrected Visual Acuity (BCVA)

A measure of a person's ability to see fine details and the best vision that can be achieved when using corrective lenses (like glasses or contacts).



Multi-Luminance Y Mobility Test (MLYMT):

A test to measure a person's ability to move through a course with 3 obstacles in a Y shape.



Multi-Luminance Shape Discrimination Test (MLSDT)

A test to measure a person's ability to identify 3 different shapes at different light levels

What Did Clinical Trials Show?

TRIAL NAME

RESTORE

PARTICIPANTS

Studied in 28 adults with advanced RP
with severe vision loss

TREATMENT GROUPS

Sonpiretigene

vs.

Usual Care

RESULTS

Outcomes for patients receiving
sonpiretigene vs. usual care:IMPROVED
vision
(BCVA)IMPROVED
to move through
Y-shaped obstacles
(MLYMT)IMPROVED
ability to identify
different shapes
(MLSDT)

These represent some, but not all outcomes that were measured in the clinical trials.

Safety of Sonpiretigene



Those who received sonpiretigene were likely to experience at least one negative visual event compared to those who received usual care. The most common negative visual events were **swelling** inside the eyes and **increased pressure** behind the eyes.

ICER's report findings are **NOT** recommendations that support the use of sonpiretigene. Patients and families should always talk with their doctors to make shared decisions about treatment for RP.

What We Still Don't Know...

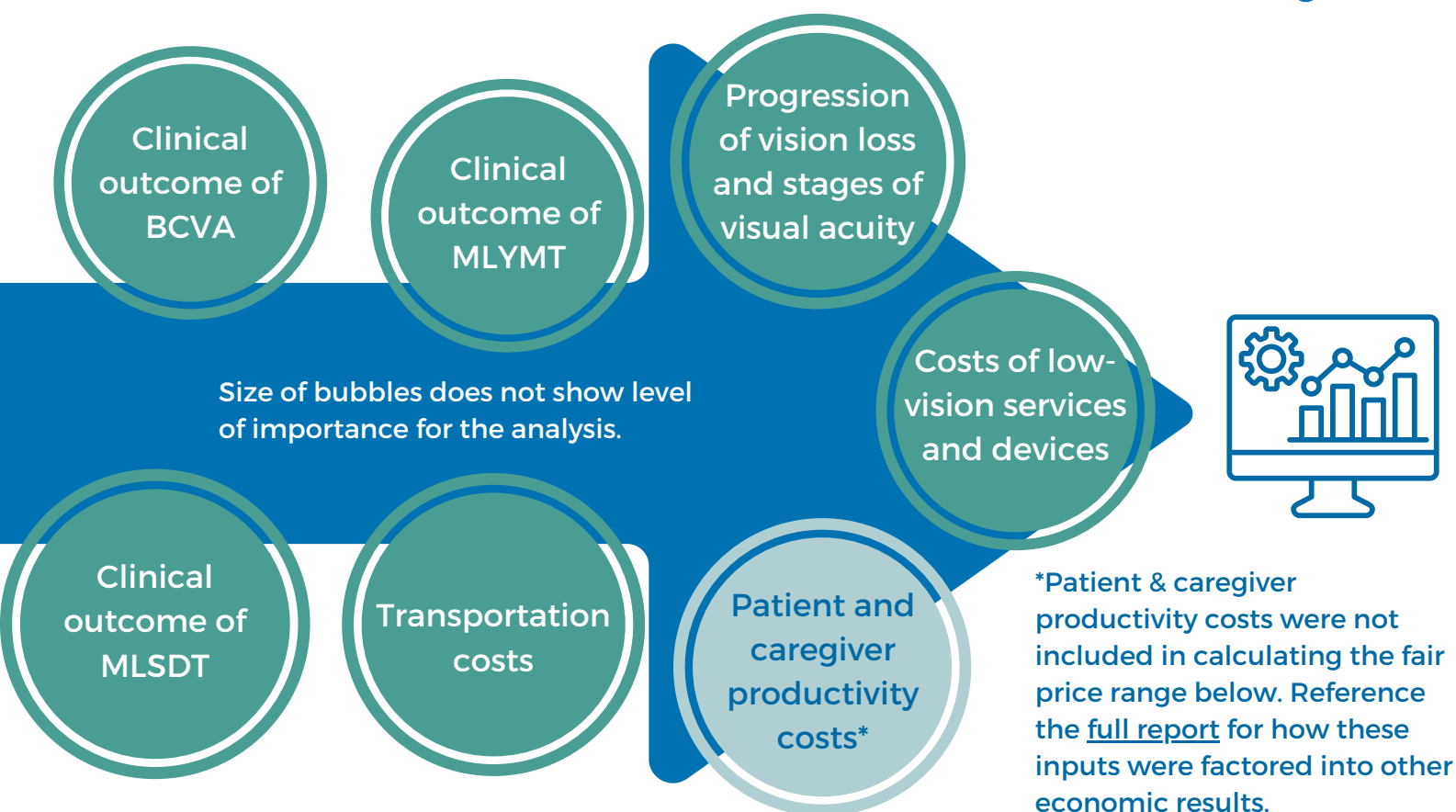
- The full range of benefits and harms from sonpiretigene
- How sonpiretigene works in the real-world due to the small trial population
- How closely the results from the trial represent true patient benefits
- How long the benefits of this one-time treatment will last

How Did ICER Calculate a Fair Price?

Using economic modeling, we calculated the cost-effectiveness of sonpiretogene based on the outcomes of **BCVA**, **MLYMT**, **MLSDT** (defined on Page 3) compared to usual care. See below for what types of information ICER considered to calculate a fair price range for this treatment.

Population:
Adult patients living with advanced RP with severe vision loss.

Factors Included in ICER's Economic Analysis



Fair Price Range for Sonpiretogene Isteparvovec

**\$67,400 to
\$101,300**
for one-time
treatment

A fair price is how much a treatment should cost based on how well it works for patients. Our economic analysis concluded that the fair-price range for sonpiretogene is between \$67,400 to \$101,300 for a one-time treatment for one eye.

Key Policy Recommendations

The Policy Roundtable at the ICER public meeting included RP patient advocates and informed several policy recommendations for pricing, access, guidelines, and future research in RP. A few key recommendations are summarized below.

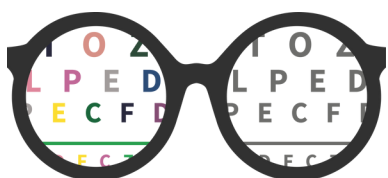
1

All stakeholders have a responsibility to make necessary accommodations for individuals with severe vision loss to have fair access to medical care.

Individuals with low vision can face unique challenges to navigating the health care system. Therefore, it is necessary to take steps to make signage in clinics, design of patient portals, and educational materials about clinical trials accessible and compatible with assistive technology.

2

Clinical specialty societies and patient organizations should educate clinicians and patients about low vision services and available rehabilitation centers.



Education for eye care specialists (especially ophthalmologists and retinal specialists), primary care providers, and patients would be crucial to promote access to these additional services.

3

Payers should expand the coverage of medically necessary services for individuals with low vision.

Assistive devices, such as white canes and magnifiers, and vision rehabilitation services for training in the use of adaptive devices, orientation and mobility, and independent living skills, including vocational adaptation are essential to the health of people with low vision.



4

Treatment can be administered by general ophthalmologists.

Due to the type of injection required for sonporetigene, general ophthalmologists can also administer this treatment. If treatment is only offered at retinal specialists or a center of excellence, then health plans should work with the manufacturer to also cover costs related to transportation and lodging.

5

Manufacturers should set prices that align with the value of their treatments.

While there is considerable hope associated with the promise of sonporetigene, there is uncertainty regarding the durability of treatment effect and longer-term safety. Manufacturer pricing should reflect these considerations in moderating launch pricing.



Impact of Patient Engagement



Patient advocacy groups **provided patient insight and data sources** on the experience of individuals living with RP, including loss of income from unemployment.



We heard patients require **constant adjustment** to new levels of visual ability and **want treatments that offer stability or maintenance**. Our economic model includes the number of years remaining with levels of vision and with light perception.



We heard that patients' ability to work was **highly connected to the level of support from their employer and the ability to manage vision loss**, which is emphasized in the report.

The Institute for Clinical and Economic Review (ICER) is an independent nonprofit organization that does research on how well new treatments work and what a fair price should be. Patients and families should always talk with their doctor to make shared decisions about the best treatment option for them.