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**Innovation for rare diseases and bioethical concerns: A thin thread between medical progress and suffering**

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### Abstract

With the development of precision medicines based on small molecules, antibodies, RNAs and gene therapy, technological innovation is providing some exciting possibilities to treat the most severe genetic diseases. However, these treatments don't always lead to a cure for the disease, and there are several factors that may hinder their overall success. Patients living during a period of great medical change and innovation may benefit from these technological advances but may also just face failures, both in terms of frustrated hopes as

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## Rare Disease by the Numbers | Innovation.org

[innovation.org/about-us/commitment/research.../rare-disease-numbers](https://innovation.org/about-us/commitment/research.../rare-disease-numbers) ▼ 翻译此页

2018年2月28日 - Today is **Rare Disease Day**, which serves as a time to recognize the more than 30 ... **progress** in advancing **innovation** for **rare diseases**, 95 percent **of** rare ... for the more than 30 million Americans **suffering from** a **rare disease** today. ... and development **challenges** to drive **innovative** options for patients.

缺少字词: **bioethical thin thread**

## Rare Disease Day: Accelerating innovation for patients in need

<https://catalyst.phrma.org/rare-disease-day-accelerating-innovation-for-patie...> ▼ 翻译此页

Conquering **rare diseases** presents a variety **of** unique **challenges** for scientists and researchers. ... to identity and the symptoms **of** the diseases are often quite different for each **patient**, ... for the more than 30 million Americans **suffering from** a **rare disease** today. ... Learn more about **progress in rare disease** research here.

缺少字词: **bioethical thin thread**

## [PDF] A Decade of Innovation in Rare Diseases - PhRMA

[phrma-docs.phrma.org/.../PhRMA-Decade-of-Innovation-Rare-Diseases.pdf](https://phrma-docs.phrma.org/.../PhRMA-Decade-of-Innovation-Rare-Diseases.pdf) ▼ 翻译此页

The Orphan Drug Act: Crucial Incentives for Continued **Advances** ... **Rare diseases** are one **of** the most scientifically complex health ... prevalence, availability **of** treatment options, and **patient** ... harming healthy cells and thereby minimizing side **effects**. In cancer .... carry blood **from** the heart to the lungs **narrow**, or become.

缺少字词: **bioethical thread**

## Consumer Updates > FDA Speeds Innovation in Rare Disease ...

<https://www.fda.gov/ForConsumers/ConsumerUpdates/ucm387513.htm> ▼ 翻译此页



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## New Report Highlights a Decade of Innovation in Rare Diseases

<https://catalyst.phrma.org/new-report-highlights-a-decade-of-innovation-in-r...> ▼ 翻译此页

Despite these challenges, we are making progress against rare disease. ... Work Remains to Address Unmet Medical Need of Rare Disease Patients. While we ...

缺少字词: bioethical thin thread suffering

## Rare Disease Day: Accelerating innovation for patients in need

<https://catalyst.phrma.org/rare-disease-day-accelerating-innovation-for-patie...> ▼ 翻译此页

... tackling these devastating and complex conditions, but how much unmet medical remains....

Conquering rare diseases presents a variety of unique challenges for ... new advances possible but for many rare diseases the causes remain elusive. ... for the more than 30 million Americans suffering from a rare disease today.

缺少字词: bioethical thin thread

## Important role of translational science in rare disease innovation ...

<https://www.ncbi.nlm.nih.gov/pubmed/25029971> ▼ 翻译此页

作者: AR Pariser - 2014 - 被引用次数: 16 - 相关文章

(1)Office of New Drugs, Rare Diseases Program, Center for Drug Evaluation ... Rare diseases play a leading role in innovation and the advancement of ... researchers, and regulatory agencies face a variety of challenges throughout the ... Rare Diseases/therapy\*; Therapies, Investigational\*; Translational Medical Research\* ...



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## Rare Disease Day: Supporting Research to Find a Rare Cure - Celgene

<https://www.celgene.com/rare-disease-day-2018/> ▼ 翻译此页

2018年2月28日 - Continued support and **medical innovation** are needed to improve the ... **advances** that have been made in the treatment of **rare diseases**, ... **challenges** and identify opportunities in the field of **rare diseases from 2020-2030**.

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## [PDF] Propelling Innovation in Drug Discovery ... - Broad Institute

<https://www.broadinstitute.org/files/sections/about/.../2012%20pcast-fda.pdf> ▼ 翻译此页

full range of **issues** where understandings from the domains of science, .... Biomedical **innovations**— including **advances** in medicines, **medical** ... Only 350 therapeutics are approved for 7000 **rare diseases** that cumulatively .... be possible for the FDA to approve drugs for **narrow** indications based on limited