

Volume 20 • Number 2

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Printed in the U.S.A.

Volume Twenty, Number Two

**PharmaVOICE** (ISSN: 1932961X) is published monthly except joint issues in July/Aug. and Nov./Dec., by PharmaLinX LLC, P.O. Box 327, Titusville, NJ 08560. **Periodicals postage paid** at Titusville, NJ 08560 and additional mailing offices.

**Postmaster:** Send address changes to PharmaVOICE, P.O. Box 292345, Kettering, OH 45429-0345.

**PharmaVOICE Coverage and Distribution:**

Domestic subscriptions are available at \$190 for one year (10 issues). Foreign subscriptions: 10 issues US\$360. Contact PharmaVOICE at P.O. Box 327, Titusville, NJ 08560. Call us at 609.730.0196 or FAX your order to 609.730.0197.

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**Their word...**



**DENISE MYSHKO**  
Managing Editor



*Strides have been made in improving cancer outcomes with the cancer death rate down 25% since its peak in 1991, making nearly one in 20 Americans a cancer survivor.*

**ROBIN ROBINSON**  
Senior Editor



*Some of the most well-known drug repurposing cases happened by accident, but today, the industry is increasingly taking a more strategic approach.*

**KIM RIBBINK**  
Features Editor



*Rare disease R&D is growing faster than any other area, bringing hope to many of the millions of patients suffering with one of around 7,000 diseases.*

## What's old is new again

This month's cover story addresses an interesting take on science, or rather repurposing scientific R&D in a different way. For a whole host of reasons — patent cliffs, increasing discovery and development costs, mergers and acquisitions — companies are becoming more strategic about repurposing existing and shelved products to build their pipelines and get as much value as possible out of their time and money investments in R&D.



According to infectious disease specialist Dr. Judy Stone in a report for Forbes, there are about 2,000 shelved drugs in the industry, including 1,500 FDA-approved drugs that were put aside because they were deemed not profitable enough. These drugs represent a ripe opportunity for companies that are eager to pursue their possibility for other indications. Developing a drug from this approach is much less costly and more time efficient. Dr. Stone says through repurposing a company could potentially develop a drug in 18 months to 36 months and for less than \$250,000.

And this strategy is not just being pursued by big pharma companies such as Astellas, Novartis, AbbVie, and Pfizer, but patient organizations, consultants, and academia are all taking steps forward to use this strategy, sometimes called rediscovery research, to find treatments from existing drugs to fill other unmet medical needs.

One of those unmet needs is in the rare disease space. Rare disease organizations have a vested interest in repurposing due to the large unmet need in the field; currently, more than 70% of rare disease patients receive off-label therapies. As such, researching the thousands of shelved or failed drugs looking for a new indication in rare disease is gaining traction in this space.

As International Rare Disease Day — Feb. 29 — approaches, it's important to remember that there are between 300 million and 400 million patients suffering from 6,000 to 7,000 different rare diseases. Yet, millions of people with rare diseases struggle to access diagnosis, treatment, and care — indeed, 95% of rare diseases do not have a single FDA approved drug treatment. In the United States alone that means more than 30 million people living with rare diseases have few to no options.

As noted in this month's Rare Disease Showcase, there is positive news. According to a 2019 study published by Tufts University, rare disease drug development is one of the fastest-growing areas in R&D, accounting for as much as one-third of products in the R&D pipeline. And in 2018, BIO reported that there were 595 companies developing orphan therapies, and almost every pharmaceutical company has, or is opening, a rare disease division.

We will be tracking #rarediseaseday, hopefully you will be too. We would be happy to share your rare story with our audience.

**Taren Grom**  
Editor

## March 2020 Patient VOICE

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