

# Understanding the Educational Needs of United States Physicians Related to Gene Therapy

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## 1 Introduction

Gene therapy, including both gene addition and gene editing approaches, is rapidly evolving and being investigated for a range of diseases. The goal of gene therapy is to alter the course of the target disease.



This study was conducted to understand current awareness, knowledge, and attitudes related to gene therapy among primary care physicians (PCPs), subspecialists, including hepatologists (hep), gastroenterologists (gastro), and endocrinologists (endo), and medical geneticists.

## 2 Methodology



A survey instrument with questions designed to understand current knowledge, experience, and attitudes with gene therapy was developed

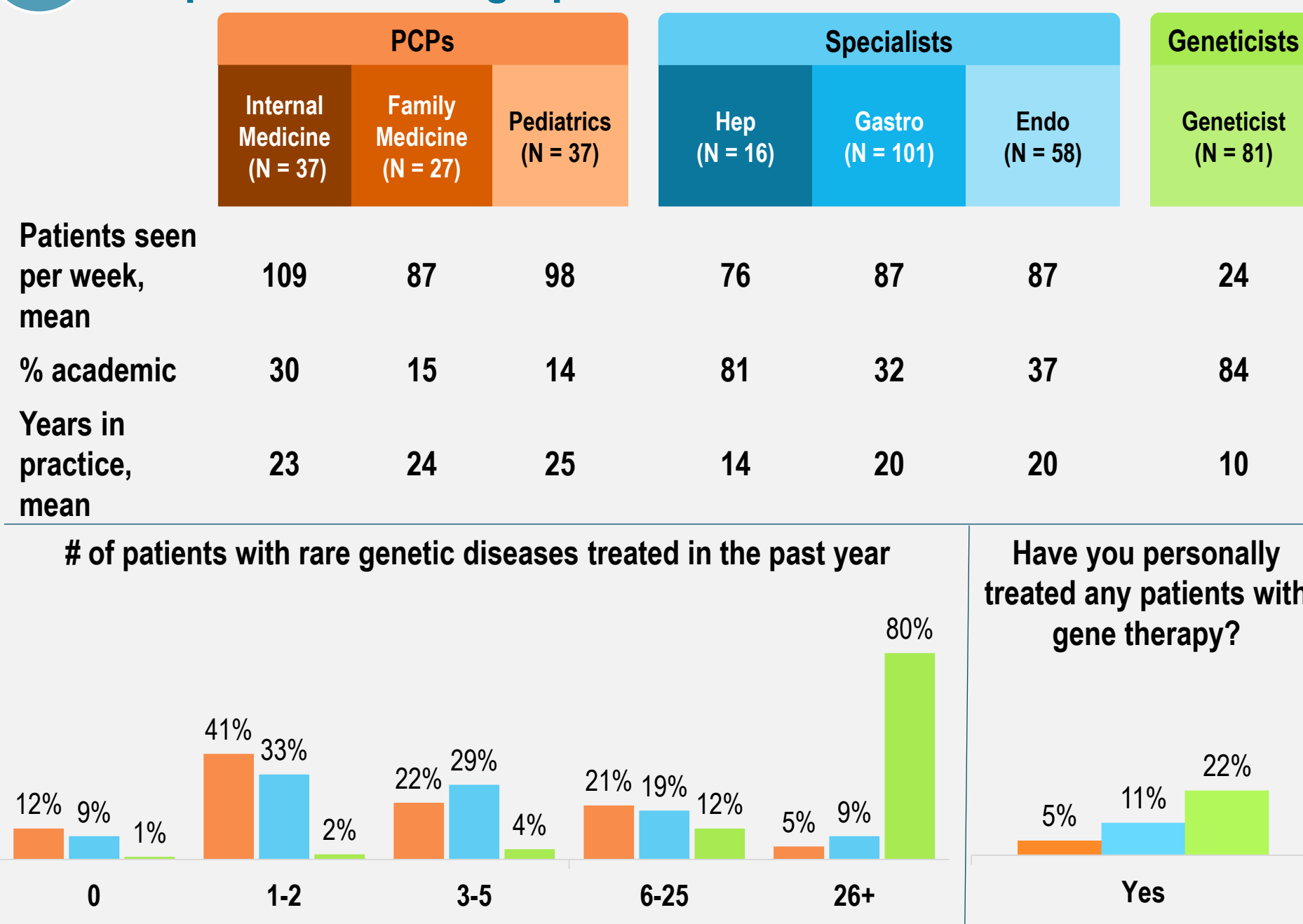


The survey was distributed via email to US-practicing physicians; responses were collected and analyzed from a total of 101 PCPs, 175 subspecialists, and 81 medical geneticists in January 2021.



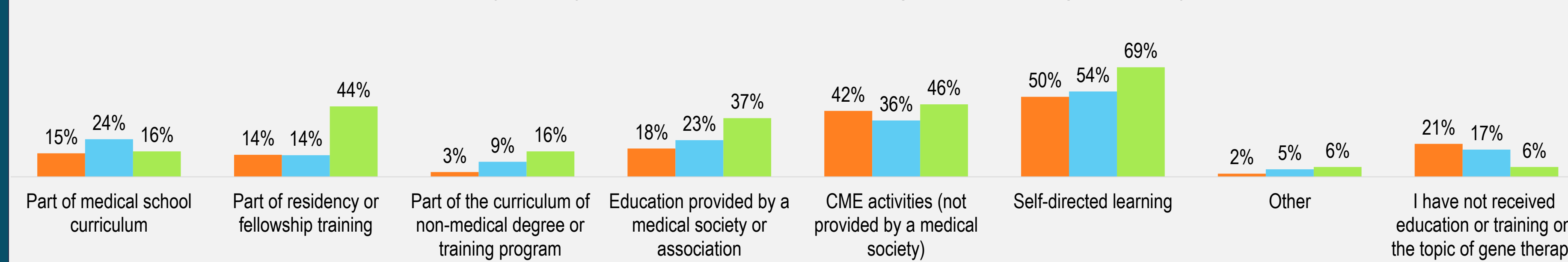
To be eligible for the survey, physicians were required to see at least one patient per week and to have treated at least one patient with a rare genetic disease at some point in their clinical practice.

## 3 Respondent Demographics



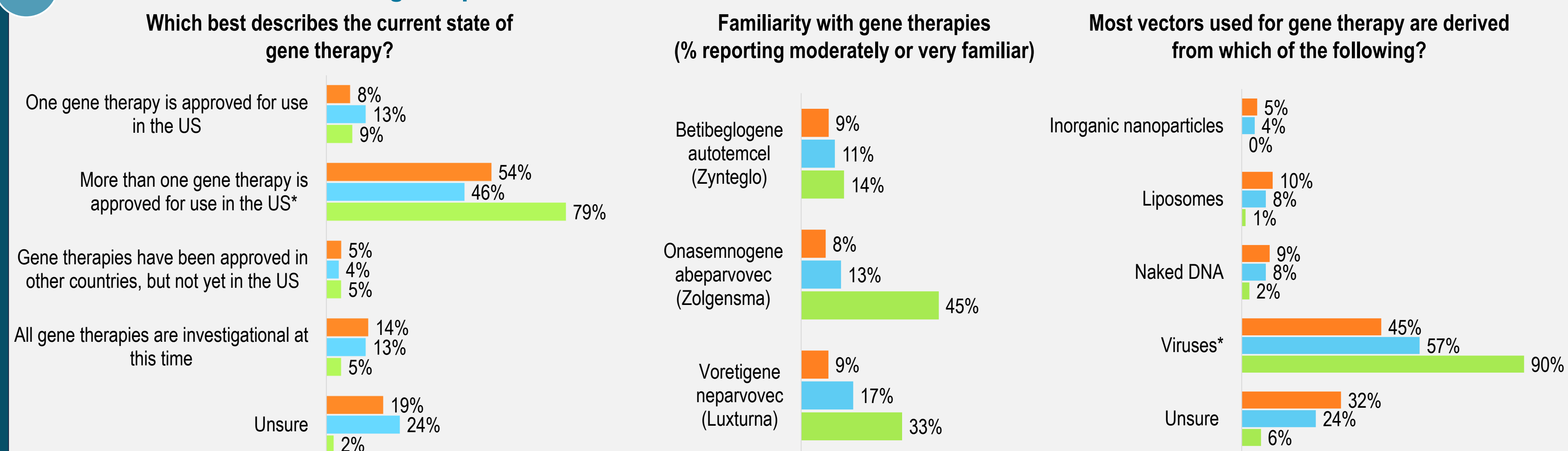
## 4 Gene Therapy Education and Training

In what ways have you received education or training on the topic of gene therapy?

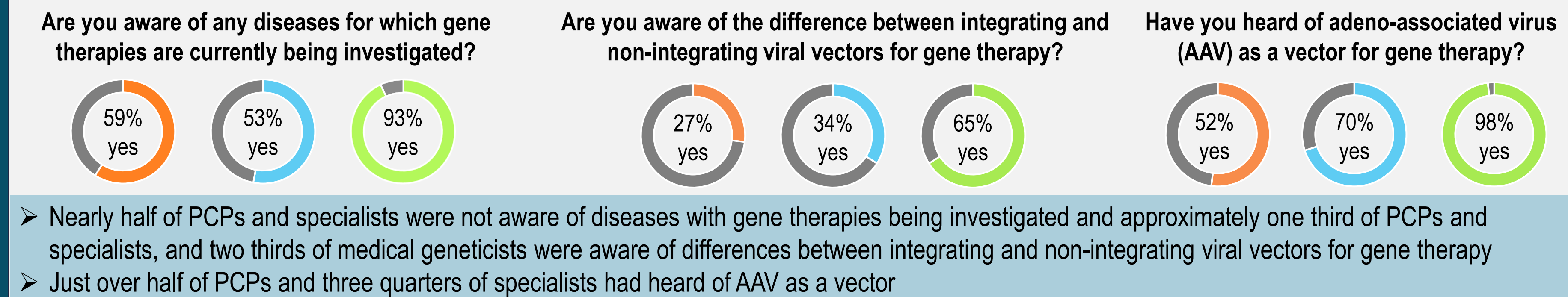


21% of PCPs and 17% of specialists never had education on gene therapy. The majority of respondents sought education on their own.

## 5 Awareness and Knowledge Gaps



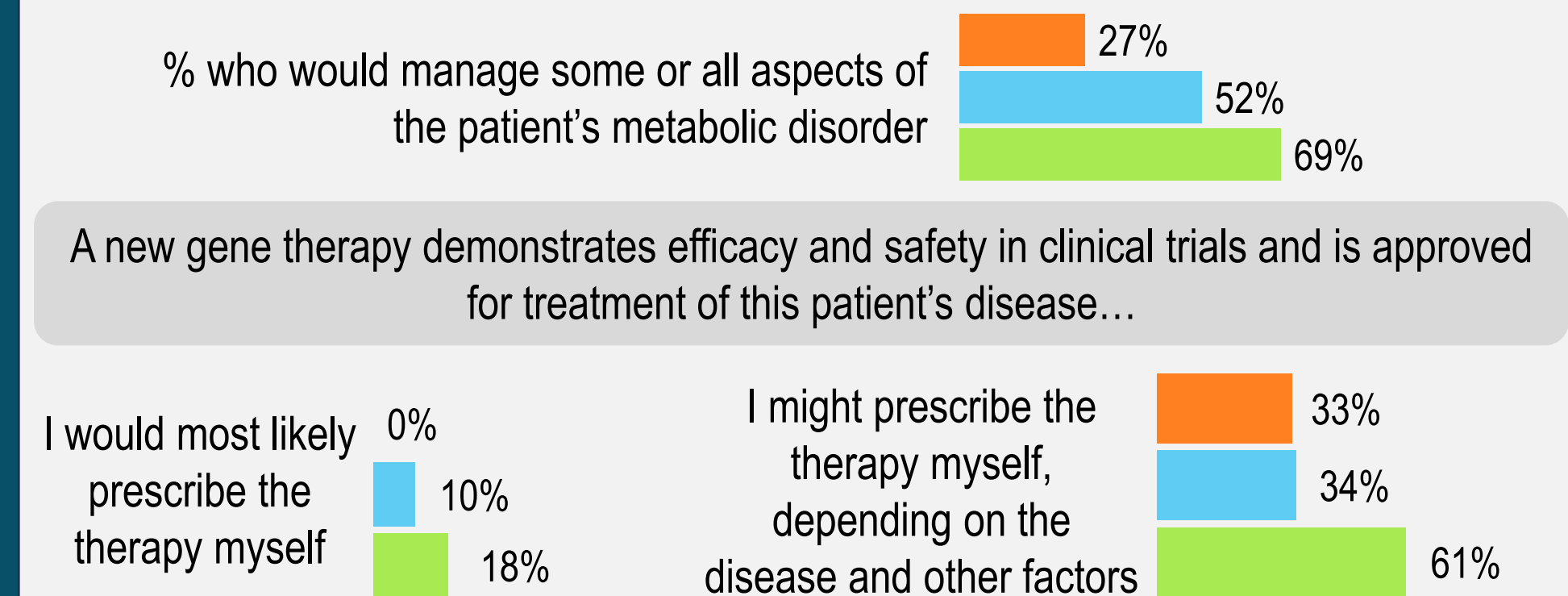
- Only about half of PCPs and specialists were aware that more than one gene therapy was approved for use in the US
- Few PCPs and specialists reported familiarity with currently approved gene therapies voretigene neparvovec and onasemnogene abeparvovec
- Most geneticists, but only about half of PCPs and specialists, were aware that most vectors for gene therapy are derived from viruses



- Nearly half of PCPs and specialists were not aware of diseases with gene therapies being investigated and approximately one third of PCPs and specialists, and two thirds of medical geneticists were aware of differences between integrating and non-integrating viral vectors for gene therapy
- Just over half of PCPs and three quarters of specialists had heard of AAV as a vector

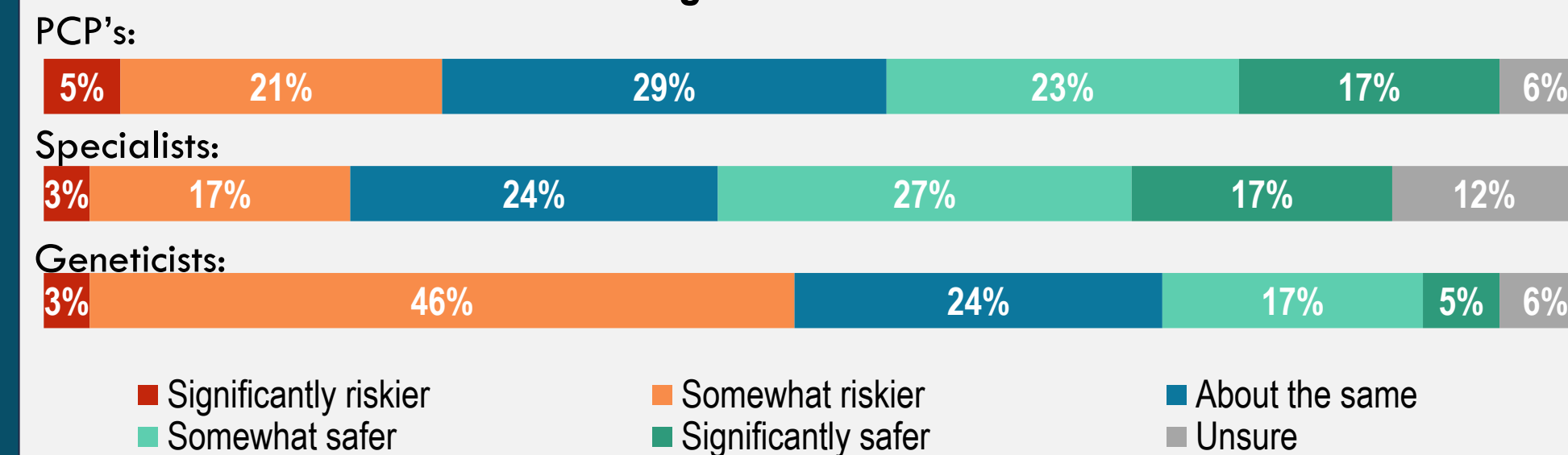
## 6 Involvement

Scenario: A medically stable patient with an inborn error of metabolism is being seen in an outpatient setting for long-term management...



## 7 Perceptions

How safe do you feel that gene therapy is compared to other treatments for genetic disorders?



## 8 Conclusions and Implications

This study highlights **gaps in awareness and knowledge** regarding gene therapy, particularly among PCPs and non-geneticist specialists. Given that most PCPs and specialists did not receive formal training on gene therapy as part of their medical training (including residency or fellowship), it is imperative that **CME initiatives address gaps in physician knowledge**. Future CME should incorporate information on the current state of gene therapy, including gene therapy approaches and processes, as well as efficacy and safety data for currently approved and emerging gene therapies.

Emily Belcher, Wendy Cerenzia, and Sylvie Stacy are employees of CE Outcomes, LLC. AnneMarie Hogan and Andrea Lacasia are employees and shareholders of Ultragenyx Pharmaceutical Inc. This research was funded by Ultragenyx Pharmaceutical Inc.