

## Commentary

# The Use of Social Media in Orphan Drug Development

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### ABSTRACT

Social media has transformed how people interact with one another through the Internet, and it has the potential to do the same for orphan drug development. Currently, social media influences the orphan drug development process in the following three ways: assisting the study of orphan diseases, increasing the awareness of orphan disease, and playing a vital role in clinical trials. However, there are some caveats to the utilization of social media, such as the need to protect patient privacy by adequately de-identifying personal health information, assuring consistent quality and representativeness of the data, and preventing the unblinding of patient group assignments. Social media has both potential for improving orphan drug development and pitfalls, but with proper oversight on the part of companies, support and participation of patients and their advocacy groups, and timely guidance from regulatory authorities, the positives outweigh the negatives for this powerful and patient-centric tool. (*Clin Ther.* 2017;■:■■■-■■■) © 2017 Elsevier HS Journals, Inc. All rights reserved.

**Key words:** disease awareness, orphan drug development, patient-centric trials, patient recruitment, rare disease, social media.

### INTRODUCTION

An orphan disease is defined as a disease or condition that affects <200,000 persons in the United States.<sup>1</sup> Even though most orphan diseases have extremely low prevalence, there are >7000 disease types in total, and collectively they affect >25 million nationwide.<sup>2</sup> There is an urgent need to develop orphan drugs that target these rare diseases with such high unmet medical need.

Historically, drug companies are reluctant to adopt these “orphan” drug development programs due to

the small target population and are more likely to focus on diseases with higher prevalence. From 1971 to 1983, there were <10 products approved for rare diseases.<sup>3</sup> This has changed substantially since the passage of the Orphan Drug Act in 1983, which provides government financial incentives to encourage companies to work in unprecedented disease areas.<sup>3</sup> Since then, there has been increasing interest in orphan drug development in the industry, with >600 orphan drug approvals received from the Food and Drug Administration (FDA).<sup>4</sup>

The nature of orphan disease, however, still poses many challenges to the drug development process. For example, low disease prevalence and highly diverse disease subgroups result in limited information available to establish an understanding of the natural history of an orphan disease.<sup>5</sup> Because even a well-informed physician may not have experience with most orphan diseases, a patient with an orphan disease might not have a correct diagnosis for years. In 2013, a survey involving 144 patients with rare disease in the United States reported that it took an average of 7.6 years to obtain the correct diagnosis, and patients typically received 2 to 3 misdiagnoses.<sup>6</sup> This low awareness of an orphan disease can result in increased patient frustration, absence of patient support, and delay in the time to treatment for patients.

In addition, low prevalence often means that patients are geographically disperse, which complicates early development planning, as well as actual recruitment during clinical trials. As drug companies aim to develop treatments for a very small patient group, understanding patient needs early in

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Social Media and Orphan Drug Development Important Events Timeline	
1983	Arpanet network architecture lays foundation for Internet
1983	President signs the Orphan Drug Act (ODA); NORD founded
1990	World Wide Web invented by Tim Berners-Lee
1992	Regulations implementing the ODA are passed
1997	First modern social network Six Degrees was launched
2005	First major video hosting and sharing site You Tube
2007	Facebook's growth is rapid with over 50 million active users and 40 billion page reviews a month
2013	FDA issues final regulations amending 1992 regulations
2016	Highest percentage (41%) of annual FDA approvals of new drugs are orphans

Figure 1. Timeline of important events in the history of social media and orphan drug development.

development becomes an imperative factor in forming company decisions compared with those working on well-understood diseases. Clinical trials for orphan diseases often differ considerably from traditional clinical trials. For example, clinical trials testing orphan drugs for rare disease indications are often smaller and allow for more flexibility in trial design than trials evaluating more common diseases. This is also because 85% of all orphan diseases are serious or life-threatening, with most lacking treatment options.<sup>7</sup> Thus, deciding on the right end point in clinical trials and designing a trial that retains patients to determine orphan drug efficacy are some of the challenges that drug companies face.

In recent years, more companies began to engage in social media, with half of the 50 largest drug makers worldwide involved in social media as of 2014.<sup>8</sup> In this article, we will discuss how social media can be utilized to overcome obstacles in orphan drug development, including raising awareness of disease, studying the natural history and progression of orphan disease, understanding patient needs, and conducting clinical

trials. We will also discuss the advantages as well as the disadvantages of employing social media techniques in orphan drug development. [Figures 1–3](#)

## DISCUSSION

### Social Media Approaches

#### History of Social Media

By 1983, a network architecture was created that would later lay the groundwork for the Internet. However, the concept of social media began to emerge in the public when the early social networking site Open Diary was founded in 1997.<sup>9</sup> The term *social media* was coined when MySpace and Facebook were created in 2003 and 2004, respectively. Web 2.0, first used in 2004, was the technological foundation that utilized the World Wide Web and provided an evolutionary platform for social media. Although both terms are used when referring to social media, Web 2.0 is considered the key technical driver, and social media entails content-sharing activities among Internet users through social networking sites accessible to certain groups that show a creative effort.<sup>9</sup> Hence, all information available on the Internet is not categorized as social media, and social media in general refers to a subset of websites or applications that allow online communities to share content.

There is insufficient information available about the natural history, disease pathogenesis, and progression of orphan diseases, which results in many poorly

#### Key Definitions

**Social media** are forms of electronic communication through which users create online communities to share information, ideas, personal messages, and other content.

**National Organization for Rare Diseases (NORD)** is a patient advocacy organization dedicated to individuals with rare diseases and the organizations that serve them.

**FindZebra.com** is a specialized search engine supporting medical professionals diagnosing rare disease.

**Orphanet** is a database providing information on thousands of rare diseases in multiple languages.

Figure 2. Key definitions.

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