



Pfizer Rare Disease

here to listen, learn and make a difference



Pfizer
Rare Disease

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February 13, 2018

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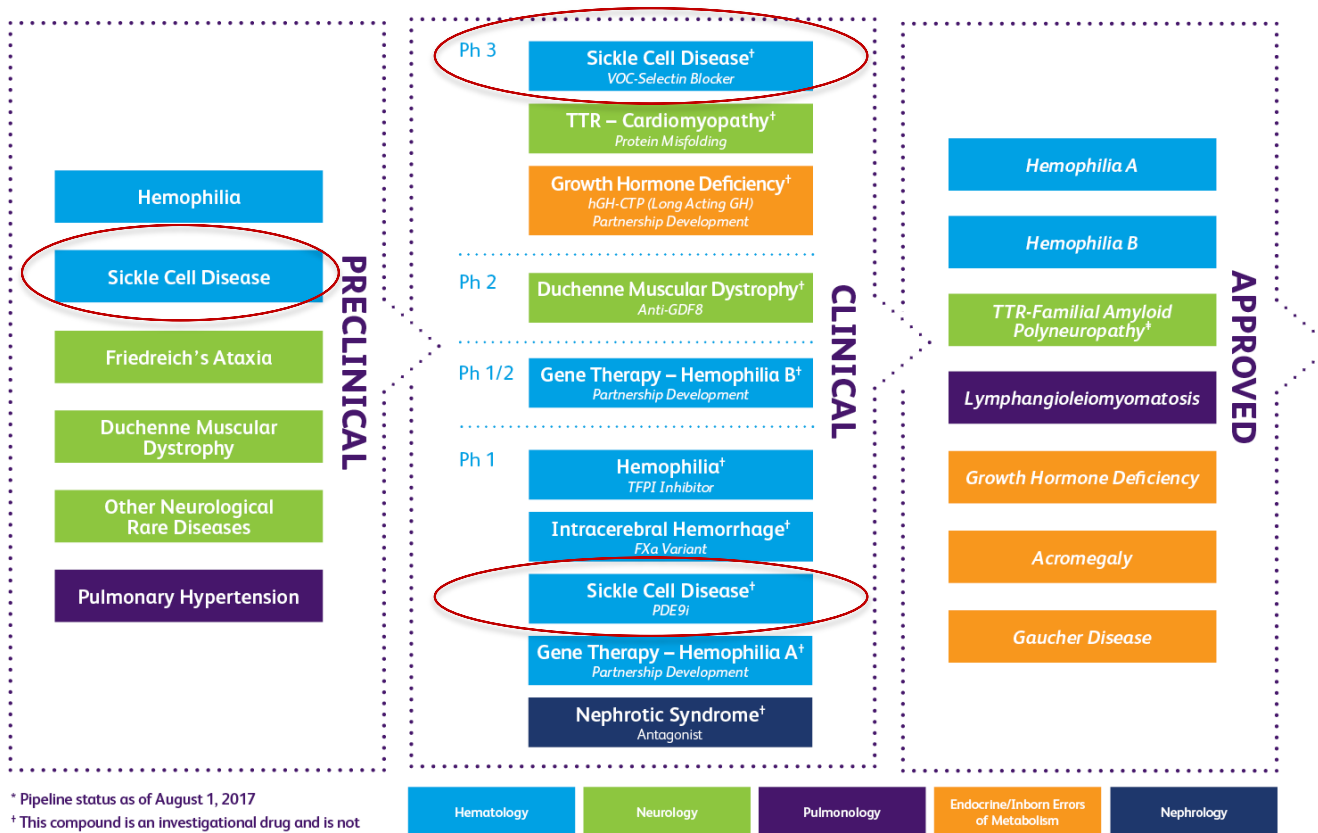
Our Commitment.

Scientists | Pfizer



- **Global footprint & investment**
- **Around 70 scientists** working to find treatments
- **Over 20 compounds** in pre-clinical and clinical development
- **Late-stage pipeline opportunities**
- **Investigational one time gene therapy treatments**

Pfizer's Global Rare Disease Portfolio*



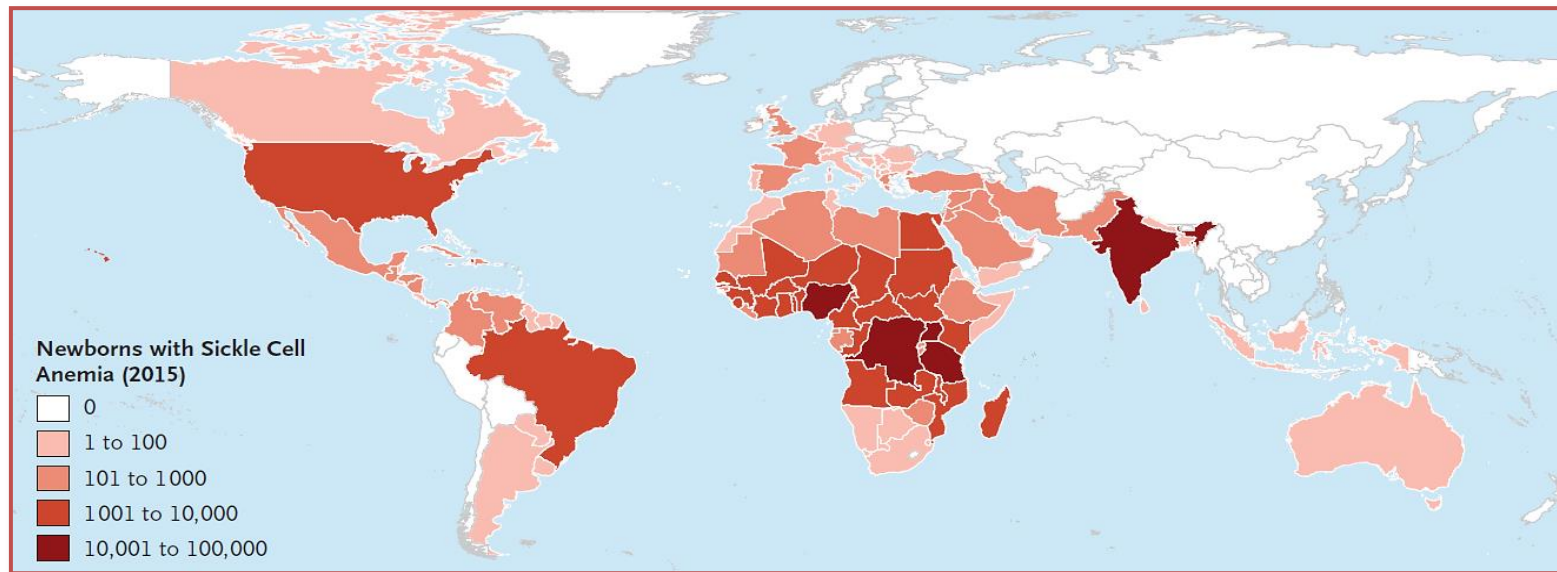
* Pipeline status as of August 1, 2017

[†] This compound is an investigational drug and is not approved by the FDA.

[†] Not approved in the United States. It is currently in registration in the United States.

Sickle Cell Disease (SCD): The Global Presence

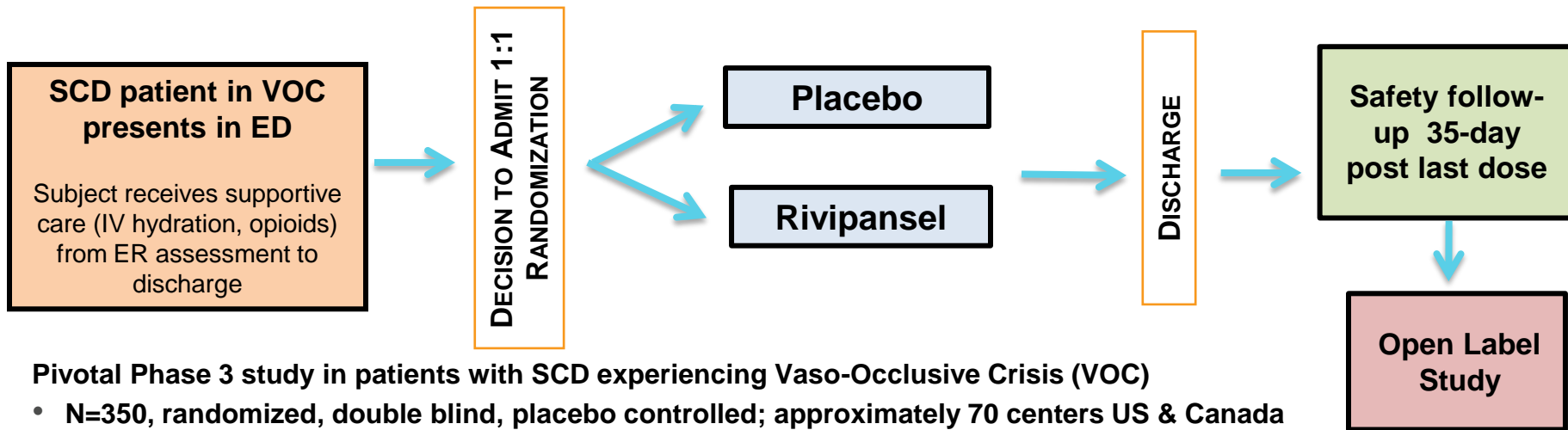
Approximately **300,000 infants worldwide** are born each year with sickle cell disease



~100,000 Americans have SCD and **2% to 10%** are **Hispanic** (~2,500–10,000)

~2 million Americans are carriers of the sick cell trait

Phase 3 Program



Pivotal Phase 3 study in patients with SCD experiencing Vaso-Occlusive Crisis (VOC)

- **N=350, randomized, double blind, placebo controlled; approximately 70 centers US & Canada**
- **Study population:** ≥6 years of age; VOC requiring hospitalization
- **Primary Endpoint:** Readiness for discharge
- **Secondary Endpoints:** Time to stopping IV opioids; cumulative IV opioid consumption

Open-label extension study

- **N=150**
- **Study population:** Subjects who complete the double-blind study and meet other qualification criteria
- **Objective:** Evaluate the safety of rivipansel when used to treat >1 episode of VOC.

Harmonizing and Empowering a Fragmented Patient Community

Empowers Patients to Self Advocate thereby Elevating the Care Paradigm

rareCourage Social Wall

Integrating vetted disease content into members' conversations



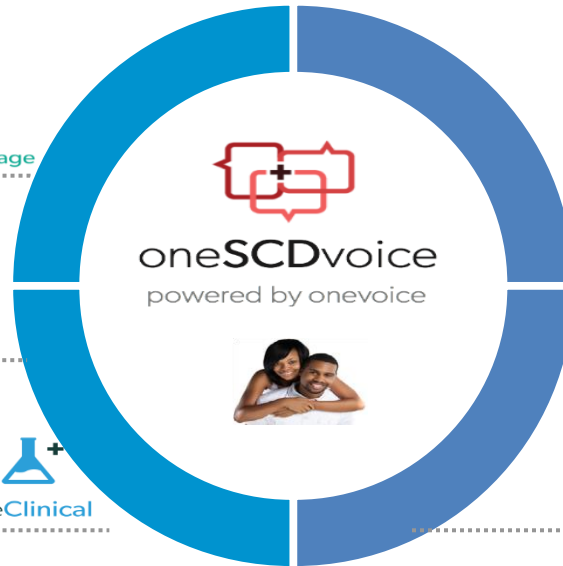
rareCurate Knowledge

Access to vetted disease content



rareClinical trials

Education for drug development and diversity in clinical trials



Curation Working Group

Guides curation of credible content to address knowledge gaps



Engagement Working Group

Drives awareness & connectivity to patients engaging local advocacy networks



Community Managers

Engages patients & advocates to utilize oneSCDvoice by providing tips & timely connections to content



Platform Developed *With* The Community and *For* The Community

Partner Organizations

Pfizer collaborates with many groups and organizations both through and beyond
“Council For Change”





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Pfizer Rare Disease is committed to transforming the lives of the rare disease community through life-changing innovations, trusted partnerships, and relentless passion.

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Time to seize the moment!

We have been here, we are here and we will be here for the Rare Disease community.



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