

# Tackling Hemophilia: Harnessing the Power of Orphan Medical Devices



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## Living with Hemophilia? You Are Not Alone

Hemophilia, a rare disease (RD), results from a clotting protein deficiency and causes uncontrolled bleeding and bruising. While it's typically a hereditary condition, it can also be acquired.

Managing hemophilia is a complex task that requires extensive treatment to stop bleeding. From avoiding injuries to controlling bleeding, the challenge is real.<sup>2</sup>

An RD is any medical condition that affects **≤1 in 2000 individuals.**<sup>1</sup>

RDs affect around **350 million people.**<sup>3</sup>

Hemophilia could potentially affect **>1.1 million people.**<sup>4</sup>

Other than the physical challenges, your quality of life is affected by issues like:

### Uncertainty



About your condition, treatments, and the future

### Isolation



Due to limited awareness in your social circle

### Unfamiliarity



With available therapeutic options

### Treatment



With frequent, painful injections

### Inaccessibility



Due to obstacles in finding specialized medical care

### Time lost



To work around lengthy treatments

Your Life, Your Challenges

## Are You Familiar with Orphan Medical Devices (OMDs)?

OMDs are products or equipment that pharmaceutical companies develop for predicting, preventing, diagnosing, supporting, treating, and managing RDs.<sup>5</sup>

## The Gap We Need to Bridge

Many patients resort to using devices that are not strictly designed for their condition.<sup>3</sup>

Clinicians have expressed the need for new disease-specific devices rather than repurposing tools intended for other diseases.<sup>7</sup>

95%

RDs have no FDA-approved treatment.<sup>6</sup>

79%

Clinicians believe genetic disorders are underdiagnosed due to a lack of diagnostic tools.<sup>7</sup>

Hurdles in the Path of OMD Development

The development of OMDs face scientific and technological challenges like:

### Limited Funding



Financial challenges due to a smaller patient population

### Regulatory Obstacles



Complex approval pathways

### Awareness Gap



Overlooked market due to lack of advocacy

You can be a catalyst in the journey of OMD development.

### Educate Yourself



Improve care and research by staying updated.

### Engage



Fuel conversations with healthcare professionals.

### Raise Awareness



Use your voice to highlight your condition.

### Connect



Patient advocacy groups can revolutionize policies.

### Participate



Contribute to innovation by getting involved in research.

### Communicate



Your needs matter. Speak up about them.

Your Role in Shaping the Landscape of OMDs

### Available OMDs for Hemophilia

- **Mobile Apps:** HemMobile, HemaGo, Factor Track, JointHealth
- **Portable coagulation monitor:** iHealth Clear
- **Smart infusion devices:** Tegu-D3 and Epi-infuse

### New OMDs under Consideration

- **Fitusiran:** Gene therapy with self-injector
- **HemaPen:** A smart medication storage and delivery system
- **Virtual Reality Training:** Self-infusion education

Shape the future by exploring opportunities through FDA, NIH, and NHF programs.

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