Medical Writing Opportunities in Rare Diseases

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Have you worked in the rare disease space?
“When you hear hoofbeats, think horses, not zebras.”
But what if it is a zebra?
Diagnosis
Before receiving a correct diagnosis, patients with rare diseases:

- see up to 8 physicians
- receive 2 to 3 misdiagnoses
- lose 5 to 7 years

Shire. Rare disease impact report: Insights from patients and the medical community. 2013.
How rare is rare disease?

• In the United States, a rare disease is defined as a condition that affects fewer than 200,000 people.

• Estimated 7,000 rare diseases

• Affect a total of 25-30 million Americans

• While each rare disease is rare individually, nearly 1 in 10 Americans is living with a rare disease!

25-30 million Americans have a rare disease
Rare diseases aren’t rare
Role of Genetics in Rare Diseases

- 80% of rare diseases are genetic in origin
- Another 20% are not
- Value of genetic testing
- Potential for treatment by gene therapy

Treatment
While more than 7,000 rare diseases have been identified, only 5 percent have treatments.

As the majority of these diseases are life threatening, these facts underscore the need for new medicines.
True or False: Writing about rare disease is bad business strategy
Orphan Drug Landscape
Financial incentives by law:

• **Exclusivity**
  - Seven years of marketing exclusivity from approval.

• **Reduced R&D costs, tax credits, and fees**
  - 50% Tax Credit on R&D Cost (decreased to 25% in 2018).
  - R&D Grants for Phase I to Phase III Clinical Trials.
  - User fees waived (FFDCA Section 526: Company WW Revenues <$50m).
Orphan Drug Designation

- US 1983
- EU 2000
- Japan 1993
What is a Rare Disease?

<table>
<thead>
<tr>
<th>Country</th>
<th>Patients/10,000 people</th>
<th>Total patients</th>
<th>Population</th>
</tr>
</thead>
<tbody>
<tr>
<td>US</td>
<td>&lt;6.37</td>
<td>&lt;200,000</td>
<td>325m</td>
</tr>
<tr>
<td>EU</td>
<td>&lt;5</td>
<td>&lt;256,000</td>
<td>512m</td>
</tr>
<tr>
<td>Japan</td>
<td>&lt;4</td>
<td>&lt;50,000</td>
<td>126m</td>
</tr>
</tbody>
</table>
>800 orphan drugs have been approved by the FDA since the passage of the Orphan Drug Act in 1983
Orphan Drug Act of 1983

Innovation in many fields of medicine, including cancer treatment and genetic diseases
Top 20 players: Everybody loves an orphan — especially when they come with blockbuster sales and a long run of exclusivity
Top 20 players: Everybody loves an orphan —

• Regulatory advantages
• Lower research costs
• Smaller studies
• Small sales force
• Trends in prescription sales of orphan drugs

• US cost of treatment, leading products with an orphan drug designation

• Key companies in the rare diseases landscape

• Orphan drug designation analysis by region and therapy area.

www.evaluate.com/OrphanDrug2019
Figure 15: Cumulative Count of Orphan Drug Designations by Region, per Year (2004-2018)
By 2024:

Worldwide Prescription Sales

$242 billion

Orphan Drugs = 20%
Vertex’s triple combination for cystic fibrosis is the most valuable orphan product with an estimated value of $24 billion.
Figure 2: Worldwide Orphan Drug Sales & Share of Prescription Drug Market (2016-2024)

+12.3% CAGR 2019-24

Orphan Drug Sales as a % of Prescription Sales

Prescription excl. generics & orphan
Generics
Orphan
Orphan drugs as a % of prescription sales
All except GSK have rare disease treatments in their pipelines
Figure 5: Worldwide Orphan Drug Prescription Sales & Share in 2024 for the Top 10 Companies

- Celgene: 5.1% Orphan, 13.7% Non-Orphan, 73% Sales from Orphan Drugs
- Johnson & Johnson: 12.7% Orphan, 33.8% Non-Orphan, 27% Sales from Orphan Drugs
- Roche: 12.7% Orphan, 33.3% Non-Orphan, 28% Sales from Orphan Drugs
- Novartis: 12.7% Orphan, 27.2% Non-Orphan, 32% Sales from Orphan Drugs
- Takeda: 11.7% Orphan, 20.0% Non-Orphan, 37% Sales from Orphan Drugs
- AbbVie: 10.1% Orphan, 25.6% Non-Orphan, 26% Sales from Orphan Drugs
- Sanofi: 7.8% Orphan, 30.8% Non-Orphan, 20% Sales from Orphan Drugs
- Vertex Pharmaceuticals: 7.8% Orphan, 7.2% Non-Orphan, 100% Sales from Orphan Drugs
- Alexion Pharmaceuticals: 7.2% Orphan, 0% Non-Orphan, 100% Sales from Orphan Drugs
- Pfizer: 6.8% Orphan, 44.7% Non-Orphan, 100% Sales from Orphan Drugs

Legend: Blue = Orphan, Green = Non-Orphan, Grey = Percentage of Sales which are from Orphan Drugs.
Dominant Orphan Drug Pipeline Candidates

- Oncology
- Cell and gene therapies
Figure 8: Worldwide Oncology Orphan Drug Sales in 2024 for the Top 10 Companies

<table>
<thead>
<tr>
<th>Company</th>
<th>Sales ($bn)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Colgene</td>
<td>12.6</td>
</tr>
<tr>
<td>AbbVie</td>
<td>9.3</td>
</tr>
<tr>
<td>Johnson &amp; Johnson</td>
<td>8.7</td>
</tr>
<tr>
<td>Novartis</td>
<td>8.2</td>
</tr>
<tr>
<td>Roche</td>
<td>7.0</td>
</tr>
<tr>
<td>AstraZeneca</td>
<td>4.0</td>
</tr>
<tr>
<td>Takeda</td>
<td>3.2</td>
</tr>
<tr>
<td>Incyte</td>
<td>2.9</td>
</tr>
<tr>
<td>Bristol-Myers Squibb</td>
<td>2.7</td>
</tr>
<tr>
<td>Eisai</td>
<td>2.6</td>
</tr>
</tbody>
</table>
Figure 6: Worldwide Non-Oncology Orphan Drug Sales in 2024 by Therapy Category for the Top 10 Companies
RetNet. Summaries of genes and loci causing retinal diseases. [https://sph.uth.edu/retnet/sum-dis.htm](https://sph.uth.edu/retnet/sum-dis.htm)
Pricing and Reimbursement Issues
Mean US cost per patient per year for top 100 products, 2014-2018

Year | 2014 | 2015 | 2016 | 2017 | 2018
--- | --- | --- | --- | --- | ---
Cost per Patient | $128,063 | $134,469 | $143,440 | $152,191 | $150,854

Pricing Issues

- Pricing depends on the marketplace (affordability, access, and innovation)

- Approved gene therapies:
  - Luxturna® $850,000
  - Zolgensma® $2.1 million
  - One-time treatments (theoretically)

- R&D cost
- Small patient pool
- Does this justify high prices?
Pricing Issues

- Reimbursement models are evolving
  - Direct sale to payer or specialty pharmacy
  - Outcomes-based rebate arrangement
  - Installment payments

- Role of ICER, NICE, etc.
FDA Orphan Drugs by Designation

- Orphan only designations: 79%
- Orphan and non-orphan designations: 21%
FDA Orphan Drugs by Designation

7 of 15 Top Selling Drugs

- Humira
- Revlimid
- Opdivo
- Enbrel
- Herceptin
- Avastin
- Remicade

Orphan and non-orphan designations 21%
Orphan only designations 79%
Patient Advocacy
Alone we are rare. Together we are strong.

280+ disease-specific organizational members
Gain insight on the needs of patients with rare diseases

RareINSERTSTATEABBREV.org
Gain insight on the needs of patients with rare diseases

• National Institutes of Health (NIH)
• Office of Rare Diseases Research / National Center for Advancing Translational Sciences (ORDR/NCATS)
• U.S. Food and Drug Administration (FDA)
• European Medicines Agency (EMA)
• European Union Committee of Experts on Rare Diseases (EUCERD)
Medical Writing Opportunities
Why consider rare diseases?

• Growing business opportunity for medical communicators
• Relatively untapped
Potential Medical Writing Clients/Employers

- Pharmaceutical and biotech companies
- Government agencies
- Advocacy groups
- News outlets
- Medical education companies
- Health insurers
- Academic institutions
- Etc.
Deliverables

• Publications
• Regulatory writing
• CME materials
• Marketing materials
• HEOR
• Meeting coverage
• Journalism
• Patient education materials
• Advocacy materials
• Grants
# Medical Writing Opportunities in Rare Disease

<table>
<thead>
<tr>
<th>Phase</th>
<th>Medical Writing Opportunities</th>
</tr>
</thead>
<tbody>
<tr>
<td>Diagnosis</td>
<td>CME, Patient Education, Advocacy, Journalism</td>
</tr>
<tr>
<td>Clinical Development</td>
<td>Regulatory (within pharma/biotech), Publications, Meeting coverage, Advocacy, Journalism</td>
</tr>
<tr>
<td>Regulatory</td>
<td>Regulatory (within agency), Advocacy</td>
</tr>
<tr>
<td>Commercial</td>
<td>Marketing materials, HEOR, Advocacy, Journalism</td>
</tr>
<tr>
<td>Post-approval surveillance</td>
<td>Regulatory (pharma/biotech and agency), Publications, Meeting coverage, Advocacy, Journalism</td>
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</table>
Therapeutic Categories

All!
Qualities and Skillsets

• Willingness to tackle a steep learning curve
• Focus on patients
• Ability to see the big picture and the small details
You’re an Expert
Resources

• FAQs About Rare Diseases. National Institutes of Health website.  


• National Organization for Rare Diseases.  www.rarediseases.org

  www.evaluate.com/OrphanDrug2019

• Endpoint News.  https://endpts.com/
Show Your Stripes!