Focus on rare diseases and uncommon cancers

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Meet Luong
What is a rare disease?
The orphan drug revolution

1983 Orphan Drug Act
<200,000 persons

1991 Singapore
“a life threatening and severely debilitating illness”

1997 Australia
<1 in 10,000

1998 South Korea
<20,000 persons

1999 Europe
<5 in 20,000

1993 Japan
<1 in 2,500

2000 Taiwan
<1 in 10,000
Orphan drug approvals in the USA

538 approval orphan drugs
(as of end of 2014)

Rare diseases: the numbers

Fastest growing sector of the biopharmaceutical industry

- There are thought to be approximately 7000 rare diseases

If all the people in the world with rare diseases lived in one country, it would be the world’s third most populous country

2. Global genes.org/rare-diseases-facts-statistics/
Challenges in orphan drug development

- Complex pathologies, with poorly defined natural history
- Difficulty identifying patients
- Small pools of experts
- Lack of awareness and political support
- Uncertainty amongst regulators
- Barriers to patient access
Forging a career in rare disease medical communications
Variety is the spice of life...

- More than 25 rare diseases in 10 years
- More than 50% were supporting the first ever treatments for patients with the condition in question
So where does Comradis fit in?

Comradis was created in 2013 to help tackle the persistent challenges facing the rare disease community.

More prompt diagnosis

Better treatments

Access for all

Ultimately, we aim to improve standards of care for those affected by rare diseases and uncommon cancers.
Com-what?

Approximately 1 in 16 people will suffer from a rare disease at some point during their lifetime.

We focus exclusively on rare diseases and uncommon cancers.

Our key areas of differentiation:
- Communications
- Commercialization
- Communities

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Re-evaluating rare disease communications

“Everything that can be invented has been invented”

Charles H Duell
Commissioner of US Patent Office, 1899
Facilitating commercialization through industry-leading insights

- Complex pathologies, with poorly defined natural history
  - Identify, evaluate and enhance sources of natural history data

- Difficulty identifying patients
  - Patient-identification strategies

- Small pools of experts
  - Stakeholder identification and mapping of current treatment practices

- Lack of awareness and political support
  - Mapping of political and legislative environment

- Uncertainty amongst regulators
  - Identification and evaluation of potential endpoint and precedents

- Barriers to patient access
  - Mapping of patient access pathways
Part of the disease community

Focus on rare diseases and uncommon cancers
Part of the disease community

Focus on rare disease research

Meet Corey
Corey is 36 years old. In 2014, he was diagnosed with Stage 4 non-small cell lung cancer ALK+ and given 6 months to live.

“Everyone was in shock,” he explains. “I had never smoked, rarely drank, had a healthy diet and I am keen cyclist and swimmer. I thought I was in great health.”

Corey’s condition is caused by a rare gene mutation that affects only 1-2% of people with his type of lung cancer. Luckily, recently developed targeted gene therapies are an option for Corey. They are not a cure, but can slow or stop cancer progression. “With two young kids and a beautiful wife, having a little bit more time means everything to me”, he says.

To learn more about rare lung cancers, visit www.rarecancer.org.au
A few recent projects...

- Patient interviews
- Patient advocacy consultancy
- Stakeholder mapping
- Portfolio planning
- Regulatory submissions
- Supporting non-profit organizations
Thoughts from a newbie

What attracted me to Comradis?

- I wanted to make a positive impact on the lives of patients who need support the most!
- I was expecting to be given a lot of responsibilities from the start
- I wanted to keep learning about rare diseases and work on a diverse range of projects ranging from consulting to medical content development

Were all of these expectations met? **YES!!!**

What skills are relevant to my role?

- Analyzing scientific data and having a good eye for detail
- Writing to the highest standard
- Having good communication skills

Alicja Busiewicz
Go into the world and do well; but more importantly, go into the world and do good

Minor Myers, Jr
Thank you

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