Better and faster drug development: Patients in the driver seat

Ana Mingorance PhD

Dracaena Consulting | www.draccon.com

@CNSdrughunter
Patient Centricity
Working for patients
Working for patients
Working with patients
Patients driving research
Why patients need to be involved in research
~7,000 rare diseases
Only 5% have an approved drug.
That is 1 in 20
10-15 new approvals per year
500 years
We need more orphan drugs
And we need them faster
Impatient Revolution
Patients are going to lead drug development. Whether pharma is ready or not.
The role of patient organizations in drug development today
In most rare diseases the science is incomplete

Disease understanding
- Limited knowledge and specialists

Know what to target
- Not known or not druggable

Preclinical path
- No animal model / translational readout

Clinical path
- New protocol / how to identify patients
Patient organizations are already leading many key scientific/medical progresses.
Patient organizations are involved in the commercial side of drug development.

Involvement in regulatory approval and national HTA

Charities unite to oppose NICE rare disease rules
March 28, 2017

Nearly 200 rare disease patient groups have come together to call on NICE and NHS England to reconsider plans for a new cost-effectiveness threshold for rare disease drugs.

Involvement of patient representatives in scientific advisory groups at EMA

Well-funded organizations become investors

Global Leader in Cure, Care, Community.

CURE DUCHENNE VENTURES

Fundamental diseases are extreme and rare genetic disorders that offer a unique opportunity to better understand human physiology and other more common conditions.

Understand and communicate the business case to pursue an indication
What needs to change by **2030** to make this better
It has to be easier for patients and companies to work together
Two worlds that are coming together

- Patient empowerment: patients make decisions on treatments, not just their doctors.
- Value-based medicine approval and pricing (PROs)
- Impatient patients: ready or not, here we go.

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<th>For 2030</th>
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Come to the dark side...

...We have cookies
3

Ultra-rare diseases and “DIY” drug discovery
They are too few.

They are not worth the investment.
They need to find a drug themselves.
Four years later, we are aware of 55 patients worldwide.

Try supplements based on that known cause (repurposing).

Starting companies to identify suitable medications.
Parent-driven innovation

FierceBiotech

Amicus spends $90M on MiaMed; gains rare disease preclinical program

by Ben Adams | Jul 7, 2016 6:52am

Life science company focused on solving Duchenne muscular dystrophy
Many of the 7000 rare diseases are ultra-rare
The majority have a genetic cause
Doctors don’t know the cause?

Find where to do gene panel / whole exome sequencing

Find genetic cause

Drug already exists?

Drug already exists?

Repurpose / off-label

Repurpose / off-label

Patient-driven drug discovery

Need to develop new drugs?

Start a company

Pharma engagement
What needs to change by **2030** to make DIY drug development possible
People want access to their own genomic data, even when uninterpretable

03-Jun-2015
Citizen-science
We need the 23andMe of DIY drug development
Access to technology platforms and approved compound libraries
Make it possible for patients to drive research
It is not only the future...
White House strategist to lead UAB’s Personalized Medicine Institute

Written by Kendra Carter

February 21, 2017

Matthew Might, Ph.D., a strategic leader appointed to the White House Precision Medicine Initiative by former President Barack Obama, has been named the inaugural director of the Hugh Kaul Personalized Medicine Institute at the University of Alabama at Birmingham School of Medicine.

Might comes to UAB from the University of Utah, where he is a Presidential Scholar and an associate professor in both computer science and pharmaceutical chemistry, and from Harvard Medical School, where he is a visiting professor of biomedical informatics. Might’s research interests focus on the intersection of computation and medicine to advance precision medicine through personalized therapeutics.

Precision medicine is an emerging practice of conducting medicine that uses a comprehensive set of resources and information — from an individual’s family history and genetic profile to lifestyle and environment — in order to guide decisions for the prevention, diagnosis and treatment of disease. It has attracted significant early attention for its promise in treating rare diseases and cancers at their root cause. The Hugh Kaul Personalized Medicine Institute, named by a gift from the Hugh Kaul Foundation, was established in 2014. Might will be the inaugural director of the institute. Nita Limdi, Pharm.D., Ph.D., has served as interim director of the institute since its creation.

Matt Might, Ph.D.
Patient Centricity today
• Patients are **experts** and thought **leaders**.
• Patient groups organize conferences and meetings, and **set the agenda**.
• They are **partners** in the development of new medicines (depends on the company)
• FDA/EMA and national regulators **consult with patient representatives** and pricing moves to value-based. Patients matter.
Patient Centricity in 2030
Patients in the driver seat

For 2030

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<th>Patients start drug development programs and then engage pharma companies just like academia does today</th>
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<td>There are services that make DIY drug discovery possible</td>
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