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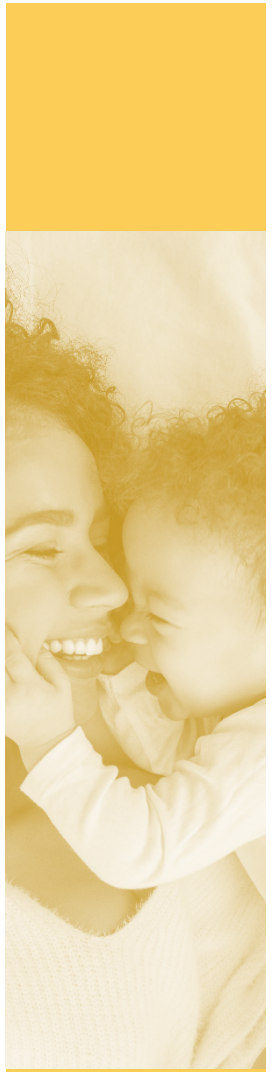
NORTHEAST BUSINESS GROUP ON HEALTH



YANN LE CAM

- Founder and Immediate Past CEO of EURORDIS – Rare Diseases Europe
- Global Affairs Advisor (volunteer) EURORDIS – Rare Diseases Europe
- EURORDIS is a non-profit alliance of over 1000 rare disease patient organizations from 74 countries that work together to improve the lives of over 30 million people living with a rare disease in Europe.

CLAUDIA HIRAWAT

- Executive Chair, VOZ Advisors
- Global consulting firm focused exclusively on patient advocacy and engagement
- Clients are global biopharmaceutical companies and nascent biotechnology companies working across therapeutic areas (including rare disorders)
- Extensive experience in rare disorders, as a patient and as former President of PTC Therapeutics, Inc.

WHAT IS A RARE DISEASE?

WHAT IS THE IMPACT?

WHAT ARE THE CHALLENGES?

WHAT ARE THE KSF?

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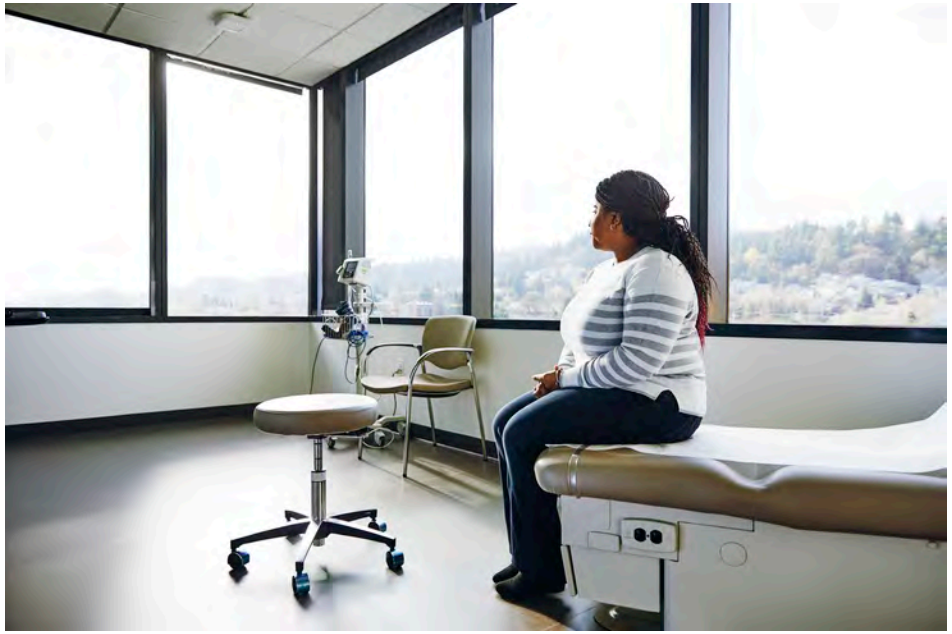
HOW RARE DISEASES ARE DEFINED?

- A rare disease is defined in the USA as a disease affecting less than 200,000 persons.
- In Europe and many other countries, it is defined as a disease affecting less than 1 in 2,000 persons (which is about the same from an employer perspective).
- There are 6,000 rare diseases (other numbers like 7,000 or 9,000 are also mentioned).



HOW MANY PEOPLE ARE AFFECTED?

- Altogether, the 6,000 rare diseases affect at least 4% of the population (range 3.5% to 5.9%, based on scientific publications)
- 300-million Persons Living With a Rare Disease in the world (PLWRD)
- 25-to-30-million Americans are living with a rare disease (based on the high range of 8% of the total population)

HOW RARE ARE THESE 6,000 RARE DISEASES?

Roughly:

- the 200 most prevalent rare diseases affect 80% of the 25-million PLWRDs in USA
- the 400 most prevalent rare diseases affect over 95% of the 25-million PLWRDs in the USA
- 5,000 rare diseases (= 85% of the 6,000) affect less than one in one-million persons or less than 350 persons in the USA

This includes a very long tail of 4,000 rare diseases, which affect only a few families (with less than five documented cases in the literature)

IN WHICH MEDICAL AREAS?

- All!
- Rare diseases affect all organs and systems, +/- 20 therapeutic areas
- 72% have a well-identified genetic cause
- 28% are non-genetic:
- Rare cancers in children and adults
  - all pediatric cancers are rare diseases and of the 220 group of cancers, 200 are rare)
- Rare infectious diseases
- Immune deficiencies
- Health hazard

ARE CHILDREN MORE AFFECTED?

- 75% of the 6,000 rare diseases affect children only, or children and adults; 25% affect adults only.
- 70% of persons of PLWRDs have the onset of their disease in childhood.
- An estimated 30% of children born with a rare disease or rare condition die before the age of 5 years.
- Sources indicate that rare diseases contribute to 55% of neonatal death; 58% of death < 1 year; 65% of death for children aged 1-14 years.

THE CHALLENGE OF THE DIAGNOSTIC ODYSSEY

- 5 years: average time for a rare disease patients to be diagnosed (up to 20 years).
- 70% of PLWRD wait more than one year to get a confirmed diagnosis after receiving medical attention.
- The key success factor is the early referral to the clinical expertise/specialized medical center for a timely and reliable diagnosis.

THE CHALLENGE OF EVOLUTIVE COMPLEX DISEASES

- Most, not all, rare diseases are:
  - evolutive and degenerative
  - complex syndromes
  - with co-morbidities over time
- Eight medical specialists from different disciplines are needed on average for each patient
- The key success factor is the regular follow up of care in a center of excellence/specialized clinic in a hospital or medical center

THE PROGRESS AND CHALLENGE OF TREATMENTS?

- Over 800 rare disease treatments (= orphan drugs) approved by the FDA in the last 25 years
- 40% of new treatments approved by FDA are for rare diseases
- Most treatment improvements are incremental, with multiple treatments for different symptoms of the rare disease and of the co-morbidity
- Emergence of curative treatments (gene and cell therapies) or disease transformative treatments (biologicals ex ERT)
- The challenge of treatment high cost for transformative or curative treatments – but high value over time
- The challenge that 90% of rare diseases don’t have any specific treatment approved

WHAT ARE THE CHALLENGES FOR EMPLOYEES AND EMPLOYERS?

WHAT ARE POSSIBLE ACTIONS?

THE CHALLENGE OF THE IMPACT ON THE FAMILY?

The challenge is that when a baby, child, teenager, adult, or elderly parent is affected by a rare disease, it has an impact on the whole family – parents, brothers and sisters, grandparents, and adult sons or daughters.

- 8 in 10 patients or care partners have difficulties completing daily tasks (household chores, shopping, preparation of meals, etc.).
- 2/3 of the family care partners spend more than two hours a day on disease-related tasks
- Three times higher need of mental health support than the general population.
- The key success factor is the daily management of medical and psycho-social care by an autonomous and empowered family.

WHAT IS THE IMPACT ON WORK?

The challenge is balancing work life and organization of care:

- 7 in 10 PLWRD and family care partners reduce or stop professional activities due to their own or their family member's rare disease
- Main care partners are mothers (64%), spouses (25%), fathers (6%)
- Absence of work:
  - <15 days/year for 42% of PLWRDs
  - >60 days/year for 25% of PLWRDs

THE UN RESOLUTION ON PLWRDS RECOGNIZE THIS CHALLENGES

The UN Resolution on Addressing the challenges of Persons Living with Rare Diseases and their Families, encourage Member States to promote access to full and productive employment and decent work, along with appropriate measures for financial inclusion for persons living with a rare disease and their families by addressing challenges:

- Access to, retention of, and return to employment, inter alia, through the creation of suitable working conditions for persons living with a rare disease and their families.
- Expanding flexible working arrangements, including through the use of new information and communications technologies.
- Providing and/or expanding leave arrangements, such as sick leave and caregiver's leave.
- Adequate social security benefits for both women and men, taking appropriate steps to ensure that they are not discriminated against when availing themselves of such benefits.

WHAT CAN EMPLOYERS DO? INFORMATION

- Raise awareness by leveraging the annual campaign of Rare Disease Day (February 28th or 29th)
- Specific information in the team where the PLWRD or care partner works.
- Ensure that health benefit providers are well versed in rare disorders and offer competitive coverage (many PLWRD and care partners switch jobs because lack of coverage).
- Have an HR department aware of rare diseases and their implications for employees – and employers.

WHAT CAN EMPLOYERS DO? FLEXIBLE ARRANGEMENTS

- Inclusiveness of PLWRDs or carers in recruitment and training.
- Working part-time.
- Flexibility over time on working full-time and part-time.
- Flexibility of X hours per year for medical appointments.
- Flexibility and follow-up on leave of absence.
- Inclusiveness on return to work.

RESOURCES

- US: National Organization for Rare Disorders (NORD) www.rarediseases.org
- Europe: EURORDIS – Rare Disease Europe www.eurordis.org
- Similar organizations exist globally in countries and regions
- Global: www.rarediseasesinternational.org
- Rare Disease Day resources: www.rarediseaseday.org
- For information assistance feel free to contact Cláudia Hirawat at CHirawat@VOZadvisors.com