PHAP
PHARMACEUTICAL & HEALTHCARE ASSOCIATION OF THE PHILIPPINES

Pharma Industry Support to Innovative Therapies for Rare Diseases

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"The State of the Rare Disease Law: Continuing Implementation and the Delivery of Responsive Health Services to the Affected Population"
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01 Industry Commitment

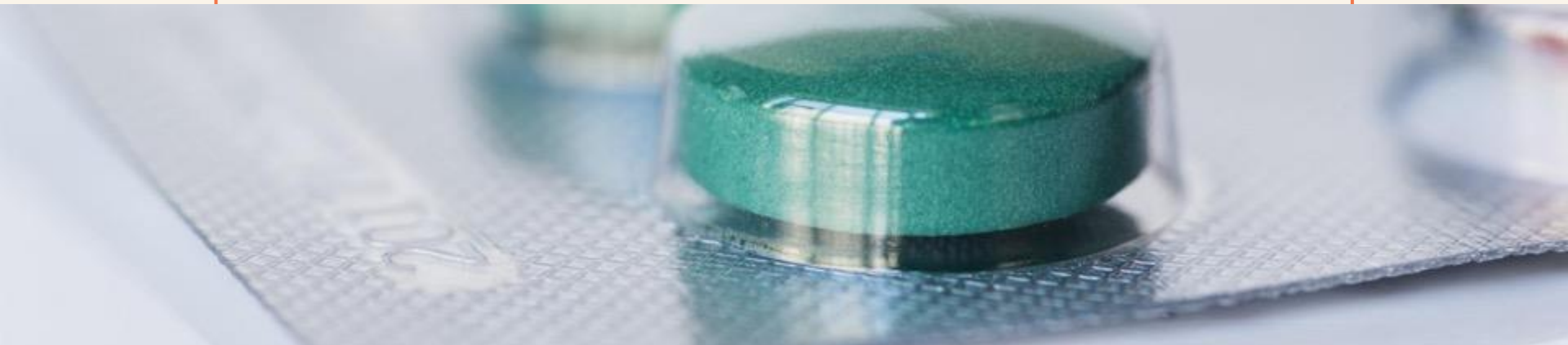
Challenges in accessing orphan drugs, and our commitment

02 Supporting Access

a. Early Access
b. Registration, CSP, and off-label use
c. Patient Access Programs
d. Building the Rare Disease Community

03 Recommendations for Greater Access

Collaboration is key. We provide a set of recommendations to facilitate access in the Philippines.



01 Industry Commitment

Accessing orphan drugs is a challenge

Only 5% of rare diseases have an approved orphan drug.

1. Regulatory challenges
    - Varying processes
    - Limited expertise
2. Access challenges
    - HTA challenges
    - Payment challenges and out-of-pocket expenses

Policy Principles to meet the challenge of Rare Diseases

- Rare Diseases as a public health priority
- Promoting continued R&D
- Empowering patients and their communities
- Ensuring sustainable patient access to diagnosis, treatment, and care

Four Strategic Pillars for Successful Product Introductions

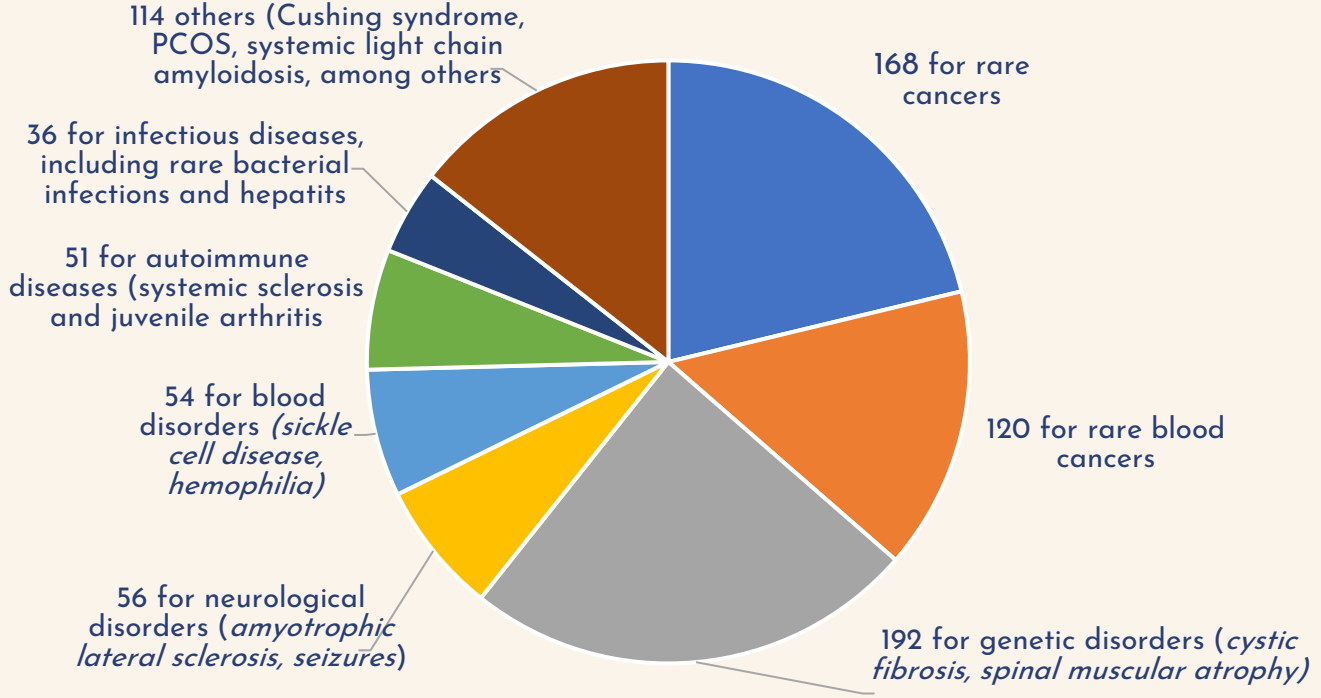
1. Commitment to the rare disease community
2. Patient group identification
3. Patient access
    - Devising early access programs to enable subsequent market availability
    - Minimizing the time between diagnosis and start of treatment
    - Helping to close potential funding gaps
4. Patient and caregivers' support

Successful launches in rare diseases, McKinsey & Company, November 2016

02
Supporting Access

The Pharma Industry Orphan Drug Research Pipeline

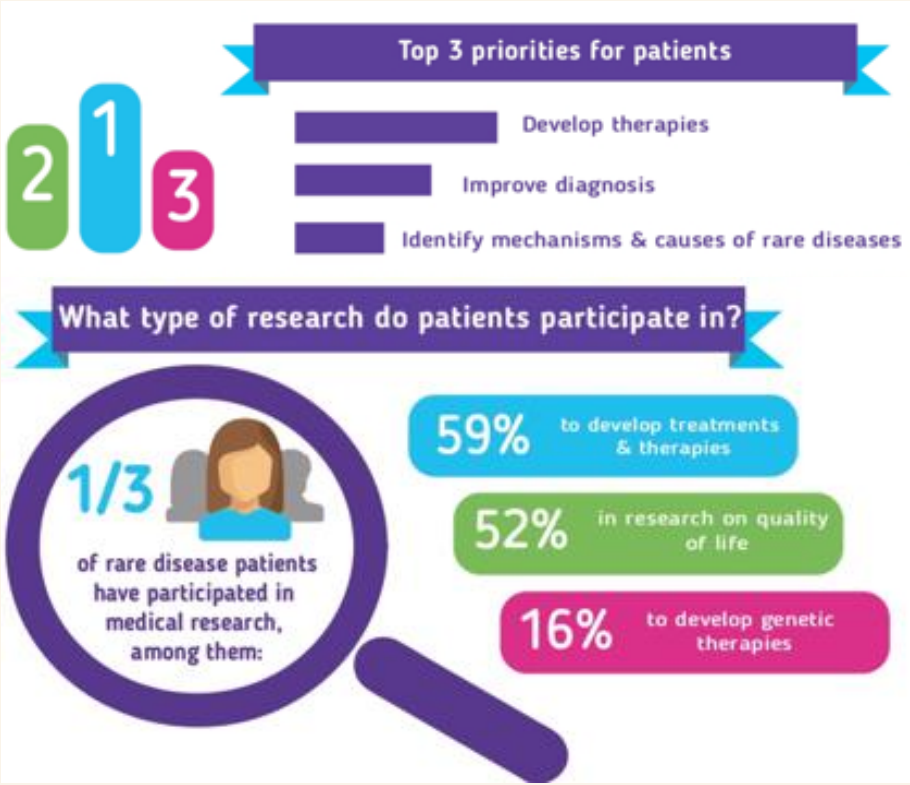
PhRMA 2021 Report on (791) Medicines in Development (All in clinical trials or awaiting US FDA review)



HOPE as scientists uncover more about rare diseases at the molecular and genetic levels, driving the development of innovative treatments for rare diseases.

Number of medicines obtained through public government and industry sources, and the Springer "AdisInsight" database; current as of November 30, 2021

Rare Disease Research



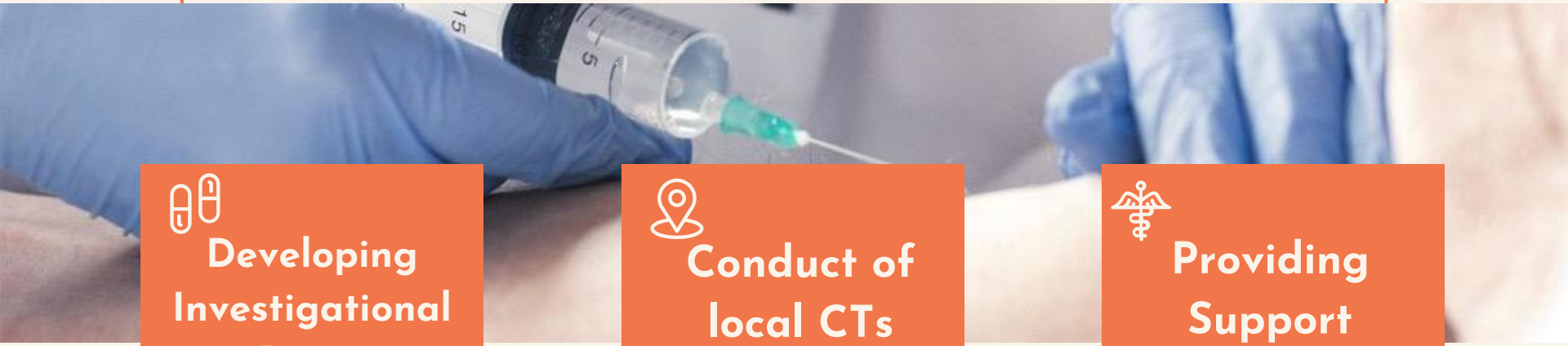
- Contributes to the development of diagnostic tools, treatments and cures, and to improved health and social care for patients and their families
- Increased over the last two decades
- Involvement by patients resulted in more research, now better targeted to their needs.

★ Patients not only reap the benefits of research but also are empowered and valued as partners from the beginning to the end of the research process.

★ Patient participation in trial = early access + post-trial support

★ Eligible Filipino patients may participate in on-going trials

PHAP Members' Activities on R&D



Developing Investigational Drugs

8 companies locally present have investigational drugs

Conduct of local CTs

2 companies have Phase III trials on rare diseases

Providing Support

Transportation support, post-trial access programs including free medicines

Registration and Compassionate Special Permits

- Securing registration or marketing authorization ensures its availability and supply.
- Existing regulatory processes are often unsuitable for rare diseases.
- Some countries do not have legislation to promote the registration and marketing approval for orphan drugs.
- When products are not registered, patients are access medicines through:
    - Compassionate Special Permit – medicines can be imported for personal use by the patient's physician from where product is approved for use. Medicines that are currently going through phase 3 trials abroad can also be accessed through CSP when no existing therapies are available. Permission is granted on a case-by-case basis
    - Off-label use – drugs that are approved for other conditions are used to treat a rare disease

Approved Orphan Drugs

8 companies locally present have globally-approved orphan drugs, available through FDA registration or CSP

Multiple sclerosis
Growth hormone disorders
Interstitial lung diseases
Rare hemophilia, chronic iron overload, non-transfusion-dependent thalassemia syndromes
Hereditary angioedemea
IBD (rare GI disease)
Glioblastoma and other brain cancers
Lipid storage disorders
Cytomegalo virus
Hunter syndrome
Congenital thrombotic Thrombocytopenic purpura
Idiopathic thrombocytopenic purpura
Metachromatic leukodystrophy
Spinal muscular atrophy
Neuromyelitis optica spectrum disorders
Huntington's disease
Paroxysmal nocturnal hemoglobinuria
Idiopathic pulmonary fibrosis
Rare cancers (NTRK, RET+ solid tumors, unresectable/metastatic melanoma & advanced NSCLC w/BRAF V600 mutation etc)
Membranous nephropathy
Lysosomal storage disorders: gaucher, pompe, mpsl and 2, fabry

Patient Access Programs

- Facilitate affordability and timely access to innovative treatments
- Enable access for patients who are not able to benefit from reimbursement schemes
- Eligibility criteria are set to ensure that the most disadvantaged benefit
- Accessed through an accredited healthcare professional, partner facility or websites

Free Treatment

Treatments for eligible patients are given free-of-charge

Special pricing

A tiered pricing program is given to eligible patients matching capacity to pay

Bundled Support

Appropriate support given to patients, such as access to healthcare professional.

Patient Access Program Examples

Novartis Philippines works with countries with advanced healthcare systems like Singapore to facilitate referral, diagnosis, and access to chimeric antigen receptor (CAR) T-cell therapy and gene therapies for patients with rare diseases.

Roche's Compassionate Use Program is a globally run access program in which eligible patients are given the medicine for free, prior to local FDA approval

Sanofi Genzyme International Charitable Access Program provides access to enzyme replacement therapies to Filipino rare disease patients enrolled in the program.
Patients are managed by the UP Institute of Human Genetics. Program has been supporting Filipino patients since 2003.

Building the Rare Disease Community

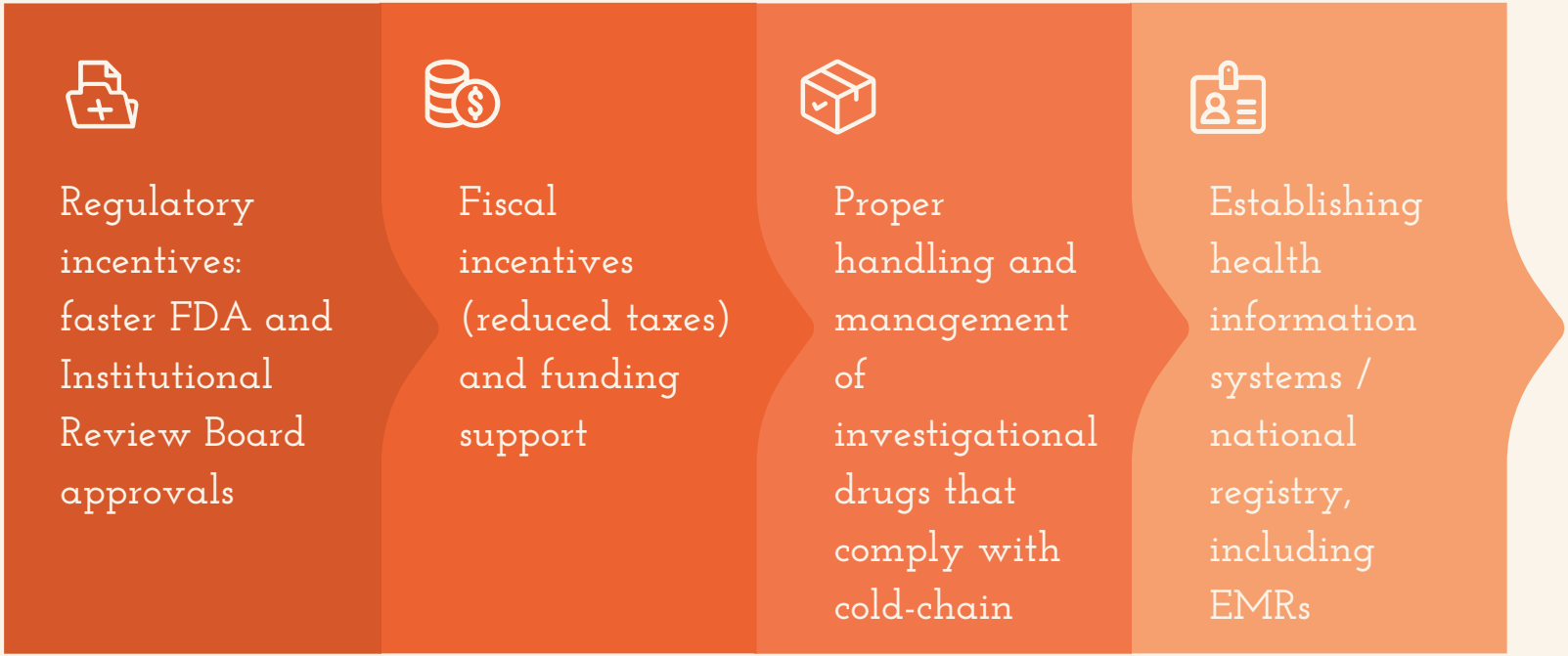
Novartis Phils.:

- 1st Asia Pacific Patient Innovation Summit (APPIS) last March 2-4 2021, virtual forum including rare diseases.
- More than 500 registrants including 377 patient leaders from almost 300 different patient organizations:
  - Common concerns of patient organizations
  - Generating solutions and capability-building to address various needs and challenges as they advocate for better healthcare outcomes
- Established the Alliance and Partnerships for Patient Innovation and Solutions

03

Recommendations

Recommendations to encourage clinical trials



Recommendations to encourage registration

- Separate pathway for FDA, HTA that will grant faster regulatory approval
- Easing regulations, providing flexibilities in the evaluation process
- Availability of government support for the treatment of patients (financing mechanism)
- Establishing health information system/ national registry that will provide data on the burden of disease

Recommendations to enable access programs

- Faster FDA donation process, including importation, customs clearance
- Fiscal and regulatory incentives
- Means to ensure sustainability, that will eventually be transitioned to a national program that industy may support
- Delinking access programs in the rationalization of government mandated price controls

Other areas of Support

- Supply chain/ logistics
- Discussion with potential suppliers (e.g. CSP)
- Rare Disease Registry
- Disease Awareness Programs

“No human life should be judged by a statistic – every rare disease patient deserves treatment and support, no matter their disease or where they live.”

-Prasanna Kumar B. Shirol
Organisation for Rare Diseases India