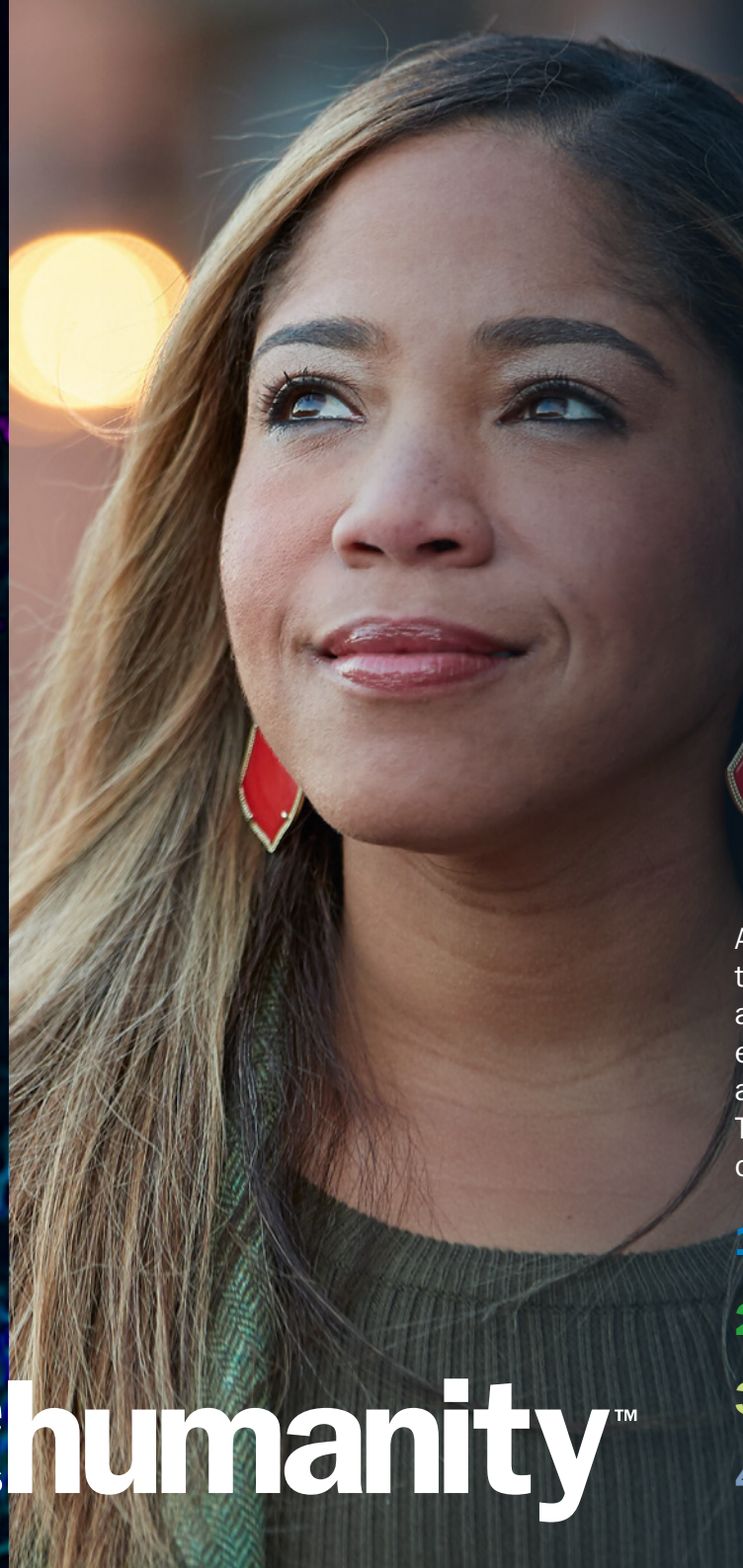
This brochure has been developed by

Biogen

Understanding **scientific** and **clinical** developments

For patients, families and caregivers



Be informed. **Be engaged.**

As new potential medicines are studied in clinical trials, you may be interested to understand more about the research and regulatory process which ensures that new medicines work well for patients and do not cause unacceptable side effects.
The following information will provide you with an overview of how drugs are developed and approved.

1 **Drug discovery**

2 **Drug development phases**

3 **Clinical trial design**

4 **Applying clinical trial data**

where science meets humanity™

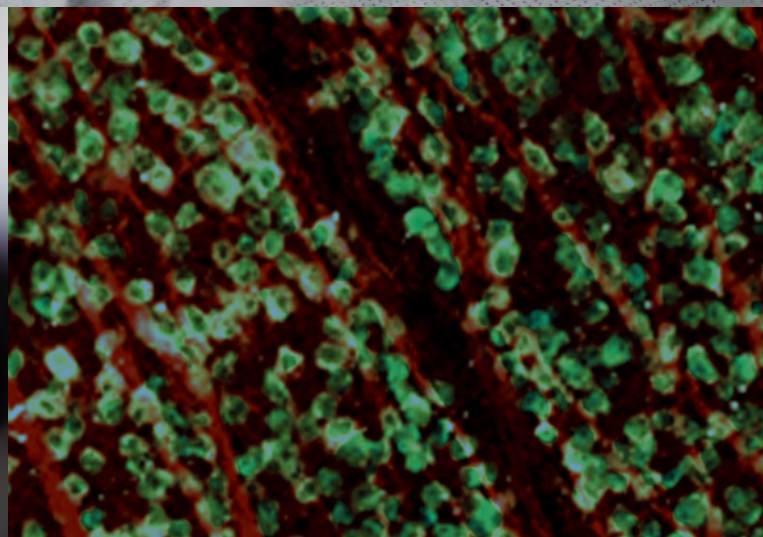
1

Drug discovery

Drug discovery is the process through which potential new medicines are identified, discovered or designed.

By understanding how a disease affects the body, scientists and researchers are able to identify drugs which can target these disease pathways.

Once a drug is identified which has potential benefit, it is first studied in the lab. If the lab experiments are successful, it is then studied in clinical trials.



2

Drug development phases

Every day, thousands of researchers, clinicians, patients, pharmaceutical companies and government agencies collaborate on clinical trials to to study drugs that are in development.

It is a very complex and time-consuming process but is essential to ensure new medicines work well for patients and do not cause unacceptable side effects. .

A new drug must pass through several stages of development called ‘phases’ to evaluate whether it can be approved for use in people and, once approved, how patients are responding to it in the ‘real-world’.

On average it takes at least 10 years for a drug to complete the journey from initial discovery to approval for widespread use.[1]

Biogen

Discovery
Identify the drug and its target within the body

▼

Pre-clinical
Investigate safety
Testing in test tubes/petri dishes and animal models

▼

Clinical Trial - Phase I
Investigate safety
20 – 80 healthy participants

▼

Clinical Trial - Phase II
Investigate safety and dosing
100 – 300 patients

▼

Clinical Trial - Phase III
Investigate safety and effectiveness
300 – 3,000 patients

▼

Clinical Trial - Phase IV
Post-approval surveillance
1,000+ patients

Clinical trials for rare diseases may have smaller numbers of participants.[2]

3

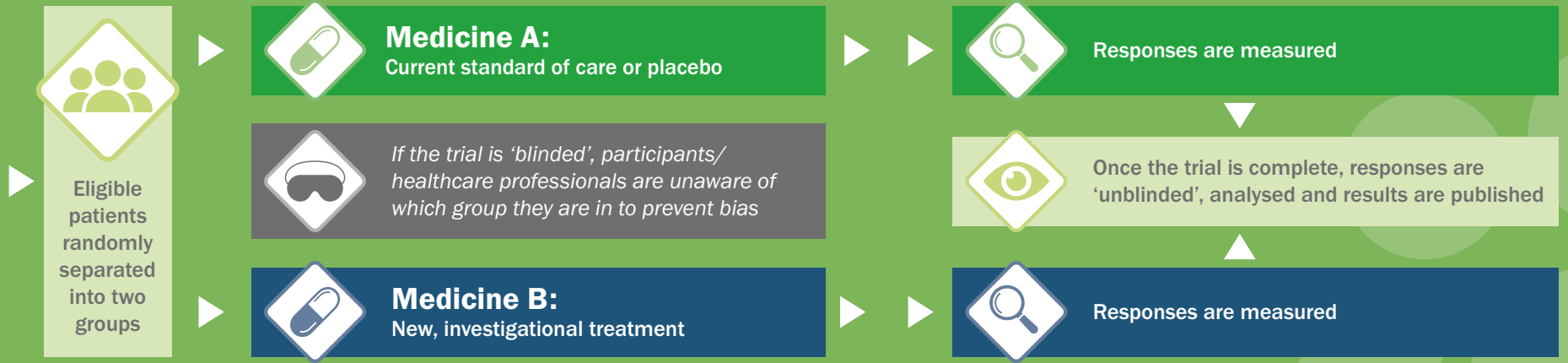
Clinical trial design

Carefully designed studies help to provide a clear picture about the potential benefit, versus risk, of giving a drug to a distinct group of people.

There are different designs of clinical trials that help to answer specific questions by the researchers. When designing a trial, a researcher will carefully consider who to include in the study. For example, will it include children or adults, people with advanced disease or early disease, people with all types of a disease, or just specific types of that disease.

They will also consider what measurements or data they will collect during the study, and whether everyone in the study will receive the same care, or if different groups will receive different care.

With rare diseases, for which there are fewer patients and sometimes no standard medicines, clinical trials often only have one group of patients. These trials compare data from patients on the investigative medicines with knowledge of how the disease progresses without any medical intervention. In these cases, the patients and healthcare professionals are generally aware of the medicines they are receiving.



4

Applying clinical trial data

Once a clinical trial is completed and the data have shown a medicine to be effective with a favourable safety profile, they can be submitted for review by regulatory authorities such as the European Medicines Agency (EMA). Based on an assessment of the data, they can approve the medicine, providing a concise statement about the purpose of the drug and how it should be used.

It is important to remember that using clinical trial data results to directly compare established medicines with those that are newly approved can be difficult as there are many variables – even if all the medicines are for the same disease, the patient characteristics and trial designs may be very different.[3] Data from clinical trials are only valuable when interpreted correctly and applied to appropriate patients.

The photographs used in this brochure are real patients and the required consent to use their pictures/stories has been obtained from the patients and families. Photographs are for illustrative purposes only.

Fast facts:

Clinical trials

- Are scientifically rigorous
- Are only of value if data are interpreted correctly
- Cannot be directly compared, even in patients with the same disease

It is important to discuss with your healthcare professional if you have any questions about clinical trials or future management of your condition.

1. Phrma, 2015. Biopharmaceutical research & development: The process behind new medicines. PhRMA.
2. Hilgers, R.D., Konig, F., Molenberghs, G. and Senn, S., 2016. Design and analysis of clinical trials for small rare disease populations. Journal of Rare Diseases Research & Treatment, 1(3):53-60.
3. The Pharmaceutical Journal. How to understand and interpret clinical data. July 2019, Vol 303, No 7927. Available at: https://pharmaceutical-journal.com/article/ld/how-to-understand-and-interpret-clinical-data (Accessed May 2021)

Biogen®

www.biogen.com

Biogen-109805. May 2021.