Clinigen's NaviGATE programme shows a third of rare disease patients are not aware that they can access unlicensed medicines

LONDON, UK, 1st February 2024 – Clinigen, the global pharmaceutical services company, has released new data at the start of Rare Disease Month that reveals while two-thirds **(68%)** of the rare community are comfortable receiving medicines before they're licensed through their country's healthcare system, one-third **(32%)** were not aware that patients can access unlicensed medicines through Early Access Programs.

These findings are from NaviGATE, Clinigen's UK-based pilot educational programme for rare disease patient advocates and organisations. The NaviGATE programme aims to equip the rare disease community with the tools they need to advocate on behalf of themselves and others more effectively. The overall goal is to facilitate greater participation in rare disease research and development and to enable increased access to medicines for more patients.

Dr Lorna Pender, Global Patient Engagement Lead at Clinigen, said: *"Shockingly, it can take five to thirty years to get a diagnosis of a rare disease, and when they finally receive one, patients often struggle to cut through the complex healthcare industry to find a way forward. Patients need to be backed by knowledge and resources, and knowing where they can get access to medicines – even before they are on the market – is vital for people with undiagnosed or rare diseases.*

"At Clinigen we have over 35 years of expertise in Early Access and 50% of our programs are focused on treatments for rare conditions. Now in collaboration with patients and patient advocacy groups, we aim to share our knowledge with the wide and deserving rare disease community through this initiative."

Rare Disease Month is recognised in February to raise awareness of the over 7,000 rare diseases that impact over 300 million people globally. NaviGATE was launched in September 2023 and these findings are the result of a questionnaire issued to the rare disease community to understand where there are knowledge gaps. Clinigen is using the learnings from the questionnaire to build an educational training programme which can serve as a resource for patients, families, community groups and organisations.

Data reveals patient advocate and organisation knowledge gaps.

The data was taken from 50 rare disease patients, parents or carers, patient advocates, organisations and charities. Of the 50, **42%** represent patients that are undiagnosed, which shows a significant white space in research and knowledge.

The data also revealed that there is a knowledge gap about unlicensed medicines. Medicines are unlicensed when they haven't been taken to market in a region, in some cases because the manufacturer decides there aren't enough patients as needed for a clinical trial, or the trial is too expensive. These medicines can be vital for patients with rare diseases.

Early Access Programmes (EAPs) can give people with life threatening or debilitating conditions early access to medicines without a commercial authorisation but where there is unmet need. Only 15

respondents **(30%)** have heard of EAPs and have a basic understanding, and only 4 respondents navigate them confidently.

36 of respondents **(74%)** would like to know which EAPs are available, and the majority **(69%)** want to know where they can access quality information about EAPs.

As many respondents to the survey reported using Google or other search engines to source information about early access to new medicines as reported asking healthcare professionals **(45%)**. Defaulting to search engines is a threat to the accuracy and safety of the information respondents are receiving.

The questionnaire has provided Clinigen with valuable insights from which to build a successful training programme to upskill and empower the rare disease patient community and brings wider awareness to the inequalities the community faces from lack of education.

Clinigen is a pioneer in the management of early access schemes and has the knowledge and expertise to address the unmet educational need among patient groups. The intention is to empower patients and patient advocates to support their communities to improve access to vital medicines.

About NaviGATE

The NaviGATE programme was built in a collaboration between Clinigen and representatives of the rare community and patient advocacy groups. The goal is to provide navigational support and tools to better engage with healthcare providers, industry and regulatory bodies. This underpins the broader mission to facilitate greater research and development into rare diseases and to enable access to medicines for more patients globally.

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Notes to Editors

About Clinigen

Clinigen is a global, specialist pharmaceutical services company focused on providing ethical access to medicines. Its mission is to accelerate access to medicines for patients in every corner of the globe. The Group supports pharmaceutical and biotech companies across the medical product lifecycle, from clinical through to commercial and operates from sites in North America, Europe, Africa and the Asia Pacific. Clinigen has more than 1,000 employees across five continents in 15 countries and provides access in more than 130 countries every year.

For more information on Clinigen, please visit http://www.clinigengroup.com.