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Outcome measures in CIDP: A scoping and mapping review

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ABSTRACT

Background: CIDP is a rare autoimmune disorder with varying symptom presentations. This heterogenous nature challenges standardizing disease monitoring and assessing treatment response. This study aimed to provide a comprehensive overview of clinical outcome assessments (COAs) used in CIDP.
Methods: A structured literature review and mapping exercise was conducted to identify COAs reported in CIDP literature published between 2010 and 2023. Supplementary searches were conducted in other relevant sources. Key publications pre-2010 were identified through handsearching or consultation with clinical experts. Clinical experts assisted with prioritization of identified COAs to those clinically relevant to CIDP.
Results: Overall, 2027 records were included, with 904 prioritized for COA mapping, and 524 reporting on relevant CIDP COAs. Overall, 99 different COAs were captured; the most frequent related to functional impairment, followed by humanistic burden. Three COAs were most prominent: INCAT, MRC score and I-RODS (reported in 45.6 %, 37.4 % and 29.2 % of publications respectively). Numerous CIDP biomarkers were identified; 21 publications reported on neurofilament light chain (NfL).
Conclusion: Substantial heterogeneity in CIDP COAs use exists. This review identified the INCAT, I-RODS, and MRC score as core measures that evaluate important, although not exhaustive, aspects of CIDP disease experience. The absence of validated CIDP biomarkers, and the heterogeneity of available COAs, may impact the assessment of CIDP treatment effectiveness. More research is needed to establish a set of comprehensive outcome measures, that are relevant to clinicians and patients with CIDP.

1. Introduction

Chronic inflammatory demyelinating polyneuropathy (CIDP) is a rare autoimmune disorder of the peripheral nervous system, characterized by peripheral nerve demyelination leading to symmetrical and proximo-distal muscular weakness, loss of tendon reflexes, and experiences of sensory disturbance [1]. CIDP pathophysiology remains unclear, and clinical presentations vary, resulting in numerous subtypes and proposed classifications [2]. For simplicity, however, the European Academy of Neurology/Peripheral Nerve Society [EAN/PNS] 2021 guideline update recommends patients with CIDP be assigned to one of two broad categories, 'typical' or 'CIDP variants', yet this results in a large degree of patient heterogeneity [2]. Regardless, the muscular weakness in CIDP, along with the associated sensory disturbance, pain, fatigue, and resulting impaired function, can limit the ability to perform daily activities and lead to impaired quality of life (QoL) and, in some cases, impact on mental health [3,4].

Clinical outcome assessments (COAs) are vital for detecting meaningful clinical changes in patients, used in both clinical research and clinical practice to support clinicians with disease monitoring and assessment of treatment response [5]. The Food and Drug Administration (FDA) describes a COA as a measure that is able to capture patient attitudes, functionality, or how a patient survives [6]. The number and variety of COAs is vast, with the four main types being clinician-reported outcomes (ClinRO), patient-reported outcomes (PRO), performance-based outcomes (PerfO), and observer-reported outcomes (ObsRO), all

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of which can be either disease-specific or generic [5]. Biomarkers are potentially useful for monitoring disease activity and treatment effects in CIDP, with markers such as neurofilament light chain [NfL] showing promise [7,8]. However, the exploration of biomarkers for CIDP remains within its early phases [9].

As recognized by working groups such as the International Consortium for Health Outcomes Measurement (ICHOM) [10] in their work in disease areas such as heart failure [11] and cancer [12], absence of outcome standardization not only complicates clinical practice assessments, but also introduces barriers for demonstrating the value of novel therapies compared with existing therapies. The lack of COA standardization and absence of measures with value to patients is an issue across many diseases of the nervous system causing muscular weakness, including myasthenia gravis (MG) and spinal muscular atrophy (SMA). Recent work has focused on complementing COAs in these disorders [13,14]. It is clear that CIDP faces similar challenges, with a need for standardized COAs that hold value to patients.

Due to differing priorities, selecting COAs that are relevant to both clinicians and patients is challenging and further complicated by the need to account for individual patient experiences and the heterogeneous nature of CIDP [2,15–17]. Previous reviews have reported on COAs in CIDP [9,17,18]. We here attempt to perform an updated, structured scoping review and mapping exercise using a broad approach to provide a comprehensive assessment of COAs used across CIDP clinical practice and research with the aim of supporting future standardization efforts.

2. Materials and methods

A structured literature search with a predefined protocol was conducted to identify outcomes used in CIDP clinical practice and research. The definition of outcome was kept broad throughout the search to ensure all relevant research was captured. Structured and comprehensive search strategies using a combination of free text and 'major subject headings' were run in Embase, Medline, and PsycINFO via OVID (Table S1-Table S3). The database searches were conducted on 28th April 2023, limited to literature published from 2010, the date of the previous EAN/PNS guidelines [19]. As CIDP is a rare disease, key publications pre-2010 were also identified separate to the database search through handsearching of recognized sources or consultation with clinical experts. In addition to the core database search, extensive supplementary literature searches were conducted in August 2023, reviewing relevant conferences, clinical trial registries, patient-reported sources, and social media from the previous two years (2021−2023) (Table S4).

A single reviewer screened the identified sources against predefined inclusion criteria (Table S5). In brief, sources were restricted to CIDP populations (based on the EAN/PNS 2021 definition), human studies, and English language publications. No restrictions on intervention or COA or biomarker type were initially applied.

COAs reported in the included sources were extracted and mapped to obtain reporting frequencies for each COA. To facilitate relative comparison between reporting frequencies, the following categories were assigned using arbitrary thresholds: Very common (≥75 publications), Common (31–74 publications), Moderately common (11–30 publications), Infrequent (3–10 publications), and Rare (≤2 publications). To aid the analysis, the identified COAs were also grouped into the three following categories according to the main aspect of the disease they aim to measure: functional impairment, humanistic impact, and 'patient/physician perceptions'. When COAs were indicated in the publication abstracts, these were used to extract the data. In instances where an abstract did not provide sufficient detail on the outcome measure used, a full text review was completed to obtain further details. Where reported, biomarkers were also mapped to provide an exploratory overview of corresponding reporting frequencies.

Clinical experts reviewed materials and provided expert guidance throughout the study to ensure COAs extracted were mapped appropriately. Due to the volume of measures captured, experts were also involved in streamlining the results to ensure only clinically relevant COAs were captured. This resulted in the development of prioritization criteria to exclude publications reporting on COAs that were rarely reported (≤2 times). All COAs excluded based on this criterion were subsequently reviewed again by the clinical experts to guarantee that no clinically relevant COAs were missed.

3. Results

A total of 2556 records were identified across the database and supplementary searches, of which 529 records were excluded due to not meeting the inclusion criteria. A total of 2027 records met the PICOTS criteria, of which 890 were prioritized for mapping (511 abstracts reporting COAs and 558 abstracts reporting biomarkers) (Fig. 1).

3.1. Overview of identified COAs

A total of 511 publications reporting COAs in CIDP were identified. COAs relating to functional impairment in CIDP were the most frequently reported in the included literature. Categories for types of COAs reported in the literature were not mutually exclusive due to publications reporting multiple COAs, therefore, the total sum of COAs from each category exceeds the total number of publications identified reporting on COAs. Assessing overall COA reporting frequency and each COA category over time showed an increase in CIDP publications reporting COAs over the past five years, peaking in 2021 (Fig. 1). The recent increase appears to be mainly driven by functional impairment COAs but also humanistic impact and patient/physician perceptions COAs.

Overall, 99 different COAs were identified, with INCAT the most frequently reported (n = 223 publications; 45.6 % of publications reporting COAs [n = 511]), followed by MRC sum score (n = 191 publications; 37.4 % of publications reporting COAs), and then I-RODS (n = 149 publications; 29.2 % of publications reporting COAs) (Table 1). These three most frequently reported COAs all belonged to the functional impairment category. Most of the COAs identified were reported in ≤2 publications (for the complete list of COA identified in the literature see Table S6).

Of the 99 COAs identified, the majority were PROs (n = 45), followed by PerfO measures (n = 24) and ClinRO measures (n = 22) (Fig. S2).

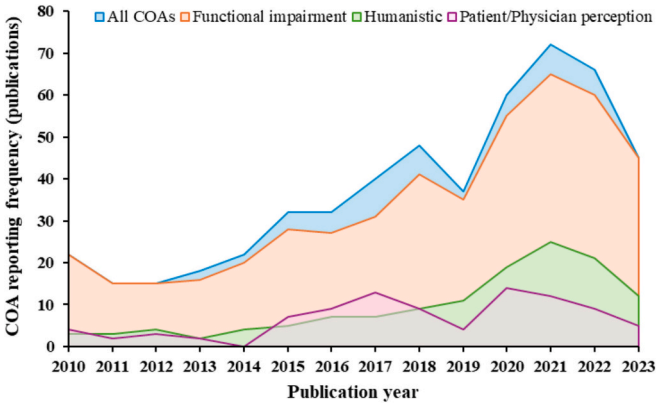

Fig. 1. Reporting frequency of COAs in CIDP, by category and year published. CIDP: chronic inflammatory demyelinating polyneuropathy; COA: clinical outcome assessment.
Note: Data displayed relate to number of publications each year and is not cumulative year-on-year or COA type (as one publication may have reported more than one COA type).

Table 1
Breakdown of reporting frequency of clinical outcome assessments (COAs) identified in the literature in at least 10 publications.

COA	Type	Reporting frequency, n	Reporting frequency, category*
Functional impairment			
Disability			
Inflammatory Neuropathy Cause and Treatment (INCAT)	ClinRO	223	**Very common**
(Inflammatory) Rasch-built Overall Disability Scale (I-RODS)	PRO	149	**Very common**
Modified Rankin Scale (mRS)	ClinRO	57	**Common**
Overall Neuropathy Limitation Scale (ONLS)	ClinRO	54	**Common**
Overall Disability sum score (ODSS)	PRO/ClinRO	38	**Common**
Neuropathy Impairment Score (NIS)	ClinRO	25	**Moderate**
9-hole peg test	PerfO	18	**Moderate**
10-m walk	PerfO	17	**Moderate**
GBS disability score	ClinRO	16	**Moderate**
40-m-walk test	PerfO	12	**Moderate**
Timed Up and Go (TUG)	PerfO	12	**Moderate**
Rotterdam Handicap Scale (RHS)	PRO	10	**Infrequent**
Muscle strength			
Medical Research Council (MRC) sum score	ClinRO	191	**Very common**
Vigorimeter measured grip strength	PerfO	40	**Common**
Jamar Grip Strength	PerfO	22	**Moderate**
Isokinetic muscle strength (cIKS)	PerfO	21	**Moderate**
Humanistic impact			
QoL			
EQ-5D	PRO	38	**Common**
SF-36	PRO	38	**Common**
Chronic Acquired Polyneuropathy Patient-Reported Index (CAPPRI)	PRO	11	**Moderate**
Pain, mood, or fatigue			
(Krupp's) Fatigue Severity Scale (FSS)	PRO	32	**Common**
Pain-Visual Analogue Scale (VAS)	PRO	16	**Moderate**
Rasch-based Fatigue Severity Scale (R-FSS)	PRO	14	**Moderate**
Beck Depression Inventory (BDI)	PRO	13	**Moderate**
Patient/Physician Perceptions			
Patient Global Impression of Change (PGIC)	PRO	11	**Moderate**

* Arbitrary thresholds: Very common (≥75 publications), Common (31–74 publications), Moderate (11–30 publications), Infrequent (3–10 publications).

Note: Only COAs with a reporting frequency of ≥10 are included in this table. See the supplementary materials for the complete list of COAs identified during the scoping review.

ClinRO: clinician-reported outcome; COA: clinical outcome assessment; EQ-5D; EuroQol-five dimension; GBS: Guillain-Barré syndrome; IVIg: intravenous immunoglobulin; N/A: not applicable; ObsRO: observer-reported outcome; PerfO: performance-based outcome; PHQ-2: patient health questionnaire-2; PRO: patient-reported outcome; SCIg: subcutaneous immunoglobulin; SF-12: 12-item short form survey; SF-36: 36-item short form survey; SF-6D: short form six-dimension; TETRAS: The Essential Tremor Rating Assessment Scale.

3.2. Functional impairment COAs

A total of 51 COAs were identified relating to functional impairment in CIDP, including two disease-specific measures, I-RODS and the Guillain-Barré syndrome (GBS) disability score. While the I-RODS was very commonly reported (149 publications), the GBS disability score was only reported in 16 publications.

INCAT for assessing disability and the MRC sum score for assessing muscle strength were very commonly reported in the literature (≥75 publications). Other measures for assessing disability, including the modified Rankin Scale (mRS), and the Overall Neuropathy Limitation Scale (ONLS), and the vigorimeter-measured grip strength for assessing muscle strength were also commonly reported (>30 publications). The Rotterdam Handicap Scale (RHS), which reflects assessment of activities of daily living, was reported infrequently (10 publications).

The majority of COAs within the functional impairment category were ClinRO or PerfO based measures. COAs that assessed sensory impairments were also included within this category yet were infrequently or rarely reported.

3.3. Humanistic impact COAs

A total of 37 COAs were identified assessing the humanistic impact of CIDP, with reporting increasing over the past five years (Fig. 1). The biggest variety in COAs identified were among those assessing QoL (n = 11 COAs), followed by COAs assessing mood, pain or fatigue (n = 19 COAs). Fewer COAs were identified assessing cognitive function (n = 5 COAs), or sleep (n = 2 COAs).

Well-known QoL measures, EuroQol-5 Dimension (EQ-5D) and the 36-Short item Form Survey (SF-36), were commonly reported (>30 publications) along with the Krupps Fatigue Severity Scale (K-FSS) assessing fatigue. Moderately reported COAs (11–30 publications) included the Pain-Visual Analogue Scale (VAS), Becks Depression Inventory (BDI), and Rasch-based Fatigue Severity Scale (RT-FSS). All these COAs have much lower reporting frequencies than the top COAs assessing disability.

Two disease-specific health-related quality of life (HRQoL) instruments were identified, and of note, in few publications only: the individualized neuromuscular QoL (INQOL) for general neuromuscular conditions (n = 3 publications), and the chronic acquired polyneuropathy patient-reported index (CAPPRI), specifically for patients with CIDP (n = 12 publications).

Most of the humanistic impact COAs were PRO measures. Four non-disease-specific ClinRO measures were identified. These were the Leeds Assessment of Neuropathic Symptoms and Signs (LANSS) pain scale assessing pain; Columbia-Suicide Severity Rating Scale (C-SSRS) assessing suicidality; the Mini-Mental State Examination; and the Neuropsychological test battery assessing patient cognition.

3.4. Patient/physician perceptions COAs

In this miscellaneous category, 11 COAs were identified: nine related to patient perceptions and preferences, one related to physician perceptions of clinical change, one related to treatment satisfaction, and one related to the use of wearable technology (e.g. smart watches). None of these measures were specific to patients with CIDP. The commonest reported was the PGIC (Patient Global Impression of Change), in 11 publications.

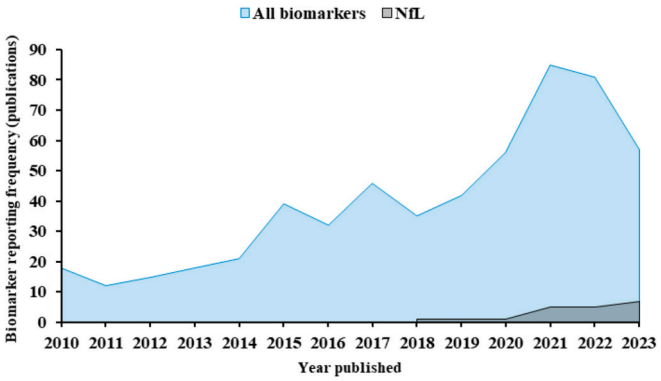

Fig. 2. Reporting frequency of biomarkers in CIDP, by year published.
CIDP: chronic inflammatory demyelinating polyneuropathy; NfL: neurofilament light chain.
Note: Data displayed relates to number of publications each year and is not cumulative.
Note: Publication year was not available for one source. The data displayed in the figure therefore relate to 557 sources.

3.5. Biomarkers

A plethora of CIDP biomarkers were identified across a total of 558 abstract and additional literature sources. In relation to publication year, a steady increase in CIDP biomarker reporting was observed over time (Fig. 2). Neurofilament light chains (NfL) was the most frequently and to date, most relevant, reported in a total of 21 sources, the majority of which were published within the last five years (n = 19). Studies reporting on NfL highlighted the potential value of NfL as a biomarker of disease activity, suggested to be reflective of axonal damage in patients with CIDP [20–23]. Notably, elevated NfL levels were reported in patients with CIDP before treatment, relative to healthy controls [20–27], which decreased after IVIg treatment and during the maintenance period [20,21,23,28], but rose again after IVIg withdrawal [21,22]. Patients with active CIDP also appeared to have higher NfL levels compared to those with inactive disease [21–23].

3.6. Discussion and expert opinion

This scoping review identified substantial heterogeneity in COAs used in CIDP, with 99 different COAs captured across 511 publications on CIDP, between 2010 and 2023. The reporting of COAs in CIDP has also accelerated over time, an observation consistent not only with increasing awareness of CIDP and the importance of disease-appropriate COAs but also the growing emphasis on COAs within regulatory and health technology assessment (HTA) applications.

COAs evaluating functional impairment represent, by far, the most commonly used measures in CIDP research and clinical practice. The INCAT and I-RODS are the most popular disability measures, together with the ONLS itself derived from the INCAT, whereas the MRC sum score and grip strength are the most utilized strength measures. INCAT, ONLS and I-RODS are all disease-specific, which represents a significant advantage in relation to relevance to the clinical characteristics of CIDP. In contrast, the available disease-specific measures evaluating humanistic burden, the CAPPRI and INQoL have remained remarkably absent from the literature, whereas general HRQoL measures, such as the EQ5D and SF-36, were much more frequently used in development programs as frequently required by regulatory bodies. Patient perceptions of change remain otherwise rarely studied to date in CIDP, as illustrated by the infrequent use of the PGIC.

Importantly, CIDP poses unique challenges that amplify the need for robust, reliable COAs. Unlike related neuromuscular disorders such as MG and SMA, which benefit from well-established diagnostic biomarkers (e.g., antibodies or specific genes), CIDP lacks validated, definitive diagnostic biomarkers [2,9,13,14]. In the absence of such biomarkers, diagnosis of CIDP is primarily based on clinical features, supported by electrophysiological and laboratory findings [1,2,9]. This reliance on clinical aspects introduces variability and subjectivity, thereby adding to diagnostic uncertainty [2,9]. This uncertainty surrounding diagnosis complicates patient classification and makes it particularly difficult to distinguish between typical and atypical CIDP variants, resulting in increased reliance on COAs to assess meaningful clinical change [9]. In this context, the question of how to trust a reported response, whether that be from a patient, clinician, or clinical trial, is key. Furthermore, in a setting where diagnostic confidence is often sub-optimal and functional change may not fully capture patient-perceived benefit, the integration of humanistic COAs could provide critical complementary insight into domains such as fatigue, emotional well-being, and daily functioning, which are areas particularly vulnerable to under-recognition in misdiagnosed or variant CIDP cases [3,4]. This challenge underscores the necessity of developing COAs that are not only valid and reproducible but also sensitive and specific to the heterogeneous CIDP population, including considerations of potential misdiagnosis [2,9].

The value of the existing commonly applied disability measures otherwise resides in their demonstrated validity and reproducibility, as well as ease of application [9,18]. While the historical emphasis on functional COAs such as INCAT and MRC sum score has offered practical, well-validated tools for assessing disability and strength, this focus has possibly limited the capacity of the field to comprehensively evaluate outcomes that matter most to patients [9,29]. Given the variability in disease progression, the challenges of diagnostic misclassification, and the fact that different symptoms can have differing impacts across individual patients, a more balanced approach integrating both functional and humanistic outcomes would likely enhance the sensitivity and relevance of outcome assessments in CIDP [2–4,9,17].

Although not considered in this review, the question of minimal clinically important difference (MCID) in the use of both disability and strength measures has gained importance in the field in recent years [29]. Despite heterogeneity of methods to determine relevant MCID cut-offs, the consideration of the concept, and its subsequent application, has brought considerable additional value to the use of these measures, in providing essential guidance both in research and in clinical practice, as to what represents change of relevance to patients [30]. It is noteworthy, however, that the optimal methods to determine MCID levels are known as “anchor-based”, where the anchor depends on patient categorization of change, determined through components of a HRQoL questionnaire or a PGIC scale. The relative scarcity of humanistic COAs in CIDP thus limits not only the measurement of patient-experienced disease burden but also the precision and applicability of MCID estimates. In a disease marked by diagnostic uncertainty, these humanistic measures could help capture subjective outcomes. That these may diverge between correctly diagnosed and misdiagnosed individuals is uncertain, but may offer insight into possible erroneous diagnosis. One example is the use of currently underutilized fatigue and pain scales, taken in context of the concurrent disability observed.

From the practical standpoint, regulatory bodies such as the FDA and European Medicine Agency (EMA) have similar approaches overall with respect to approval based on disability measures. However, it is noteworthy that PRO-based labels as a whole are more likely to be approved by the EMA (47 % of labels approved by the EMA between 2006 and 2010 included a PRO label claim compared with 19 % of those approved by the FDA) [31,32]. Of note, as outcomes are often agreed with regulatory bodies beforehand, it is possible that this may limit the willingness and ability for researchers to derive, propose and use new outcomes, however valuable. The continuing predominance of the INCAT scale in studies to date, despite other arguably more adequate scales becoming available, illustrates this. Emerging CIDP therapies will likely rely on a combination of COA types, and a lack of consistency

between United States and European regulatory bodies may introduce further complexities in relation to COA standardization in CIDP.

Despite the large number of proposed biomarkers, expanding exponentially in recent years, only NfL has consistently demonstrated potential value in CIDP to date. Nevertheless, biomarkers in CIDP remain early in development and are unlikely to replace COAs as primary measures of clinical benefit. Instead, biomarkers may serve as valuable adjuncts to COAs, complementing the direct assessment of disability and quality of life that matter most to patients. Collectively, these challenges and opportunities underscore the importance of standardizing COA frameworks in CIDP, refining MCID thresholds using patient-centered anchors, and rigorously evaluating how outcome measures perform across both diagnostically certain and uncertain patient populations.

4. Conclusion

This study identified substantial heterogeneity in relation to CIDP COAs, although INCAT, I-RODS, and MRC are among those COAs more commonly used. Given the diagnostic ambiguity and heterogeneous clinical presentation of CIDP, developing a standardized, multidimensional set of COAs tailored to both typical and variant forms of the disease is desirable. Similar frameworks have been successfully established in other neuromuscular disorders, such as MG, and could serve as useful models [33].

A critical consideration for future COA development is the need to capture the full spectrum of disease impact, including humanistic burden and patient-reported perspectives, which are often overlooked by traditional functional measures. This is particularly important in cases where there is diagnostic uncertainty and subjective patient experiences may offer insightful supplementary information, especially when functional changes are unclear or inconsistent with PROs. Further research is required to assess the place and relative value of existing COAs, their combined use, and importantly, to determine appropriate MCID thresholds. Such work should explicitly account for how COAs perform in correctly diagnosed versus misdiagnosed patients, given the known diagnostic challenges in CIDP. The importance of integrating patient-centered, humanistic measures in CIDP outcome assessment cannot be understated, as they may capture critical domains of HRQoL and patient experience that functional measures may overlook. Although the biomarker landscape is expanding, with NfL showing preliminary promise, biomarkers are unlikely to replace COAs as primary efficacy measures, but may provide valuable complementary information when combined with functional and humanistic assessments.

CRediT authorship contribution statement

Yusuf A. Rajabally: Writing – review & editing, Supervision. **Giorgio Maria Boggia:** Writing – review & editing, Supervision, Methodology, Investigation, Funding acquisition, Data curation, Conceptualization. **Danielle Riley:** Writing – review & editing, Writing – original draft, Visualization, Validation, Supervision, Resources, Project administration, Methodology, Investigation, Formal analysis, Data curation, Conceptualization. **Sam Riley:** Writing – review & editing, Writing – original draft, Visualization, Validation, Supervision, Methodology, Investigation, Formal analysis, Data curation, Conceptualization. **Judith Peatman:** Writing – review & editing, Writing – original draft, Visualization, Validation, Supervision, Methodology, Investigation, Formal analysis, Data curation, Conceptualization. **Wim Noel:** Writing – review & editing, Supervision, Methodology, Investigation, Funding acquisition, Data curation, Conceptualization. **Charlotte Gary:** Writing – review & editing, Supervision, Methodology, Investigation, Funding acquisition, Data curation. **Eduardo Nobile-Orazio:** Writing – review & editing, Methodology, Investigation, Conceptualization.

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Declaration of competing interest

Y. A. Rajabally has received speaker/consultancy honoraria from LFB, Polyneuron, Argenx, Takeda, Janssen, Sanofi, Dianthus, Grifols, Vitaccess, has received educational sponsorships from LFB and CSL Behring and has obtained research grants from LFB.

E. Nobile-Orazio has received speaker/consultancy honoraria from Argenx, Takeda, CSL Behring, Dianthus, Janssen, Kedrion, LFB, Roche and has received a research grant from Takeda.

G. M. Boggia, C. Gary, and W. Noel are employees of Johnson and Johnson.

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Appendix A. Supplementary data

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Data availability

There is no associated data set.

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