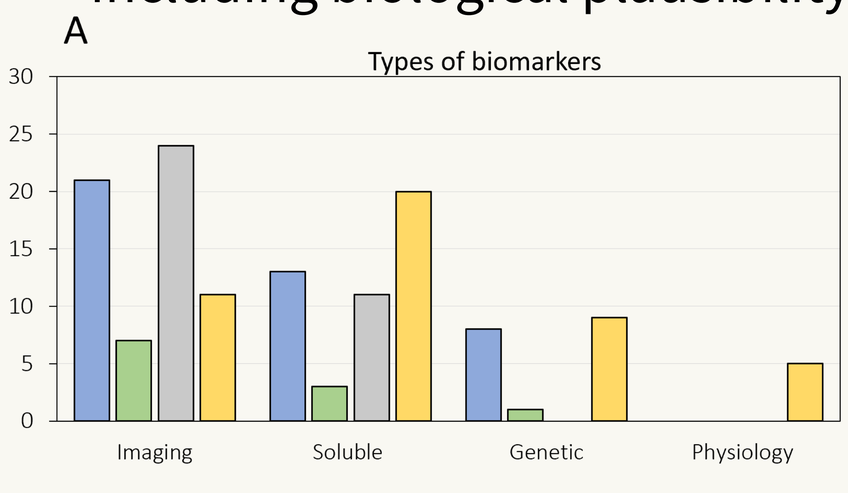
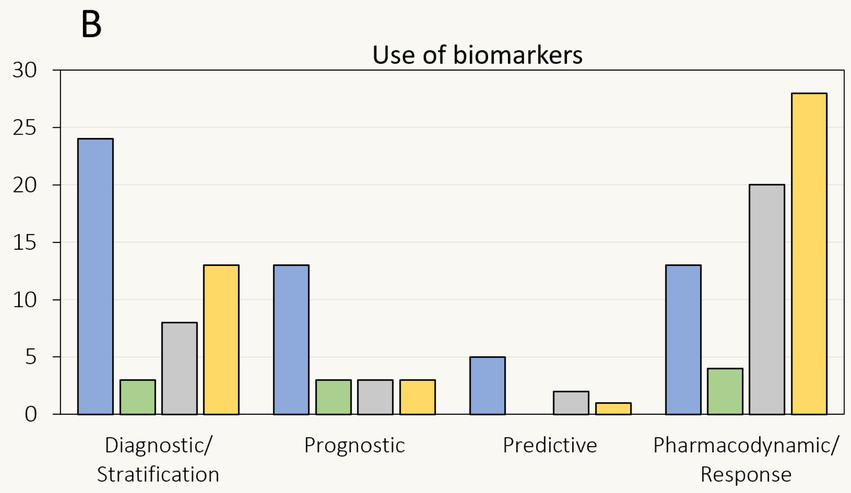
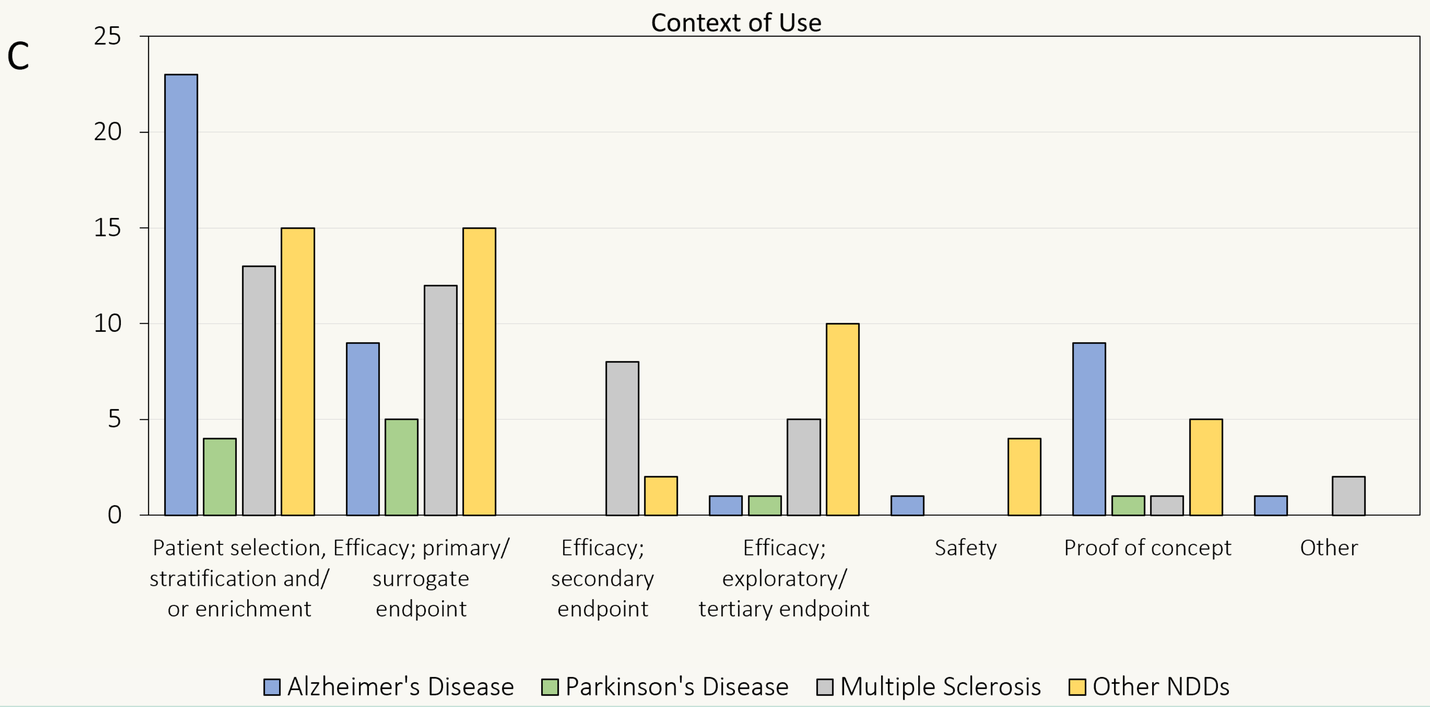
To successfully implement novel biomarkers for neurodegenerative diseases a robust evidence base is required

Including biological plausibility or correlation between biomarker and meaningful clinical benefits







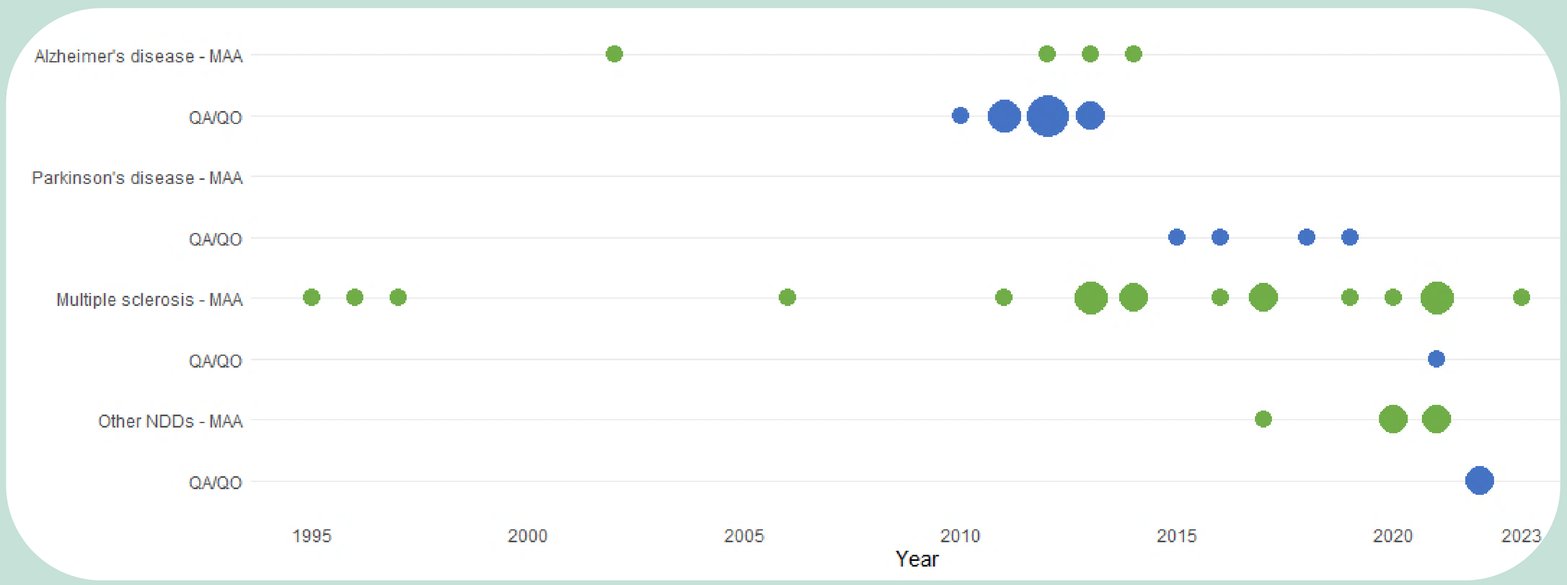
Number of times *Types of biomarkers* (A), *Use of biomarkers* (B) and *Context of Use* (C) were seen in the analysed MAAs, QAs/QOs, and SAs for Alzheimer's Disease, Parkinson's Disease, Multiple Sclerosis, and other NDDs.

Biomarkers can be valuable in the development of treatments for Neurodegenerative Diseases (NDDs), but no comprehensive overview was available that details the role of biomarkers in the development, and European regulatory advice- and decision-making, for NDD treatments.

RESULTS

The acceptance and role of biomarkers differs between AD, PD, MS and other disease areas.

In the regulatory documents we saw that biomarkers on their own were not sufficient to confirm AD diagnosis and that early interaction through the QA/QO procedures was sought most frequently in AD. We did not find any MAAs for new active substances for PD that discussed biomarkers. Lesion imaging biomarkers for MS have an established role in the regulatory dossiers and clinical guidelines. Also, the majority of SAs discussing biomarkers were in the context of other NDDs.



Number of MAAs (1995-2023) and QAs/QOs (2008-2023) that discuss biomarkers for the different diseases over time.

METHODS

We analysed the EMA's MAAs, QAs/QOs and SAs regarding NDDs that mention biomarkers

In total, 105 procedures involving NDDs that discussed biomarkers were analysed; 57 SAs (Jan 2020 – Dec 2022), 19 QAs/QOs (Jan 2008 – Dec 2023), and 29 MAAs (Jan 1995 - Dec 2023). Most of the procedures involved: Alzheimer's Disease (AD; n= 30); Parkinson's Disease (PD; n=9); Multiple Sclerosis (MS; n=33).

CONCLUSION

Despite the established role of some biomarkers in regulatory dossiers and clinical guidelines, more precise and reliable biomarkers for NDDs are essential to enhance diagnostic accuracy, and disease and treatment monitoring.

Abbreviations:

NDD:	Neurodegenerative Disease	QA:	Qualification Advice	AD:	Alzheimer's Disease
EMA:	European Medicines Agency	QO:	Qualification Opinion	PD:	Parkinson's Disease
MAA:	Marketing Authorisation Application	SA:	Scientific Advice	MS:	Multiple Sclerosis

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Biomarkers for Neurodegenerative Diseases in Regulatory Decision-making by the European Medicines Agency

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