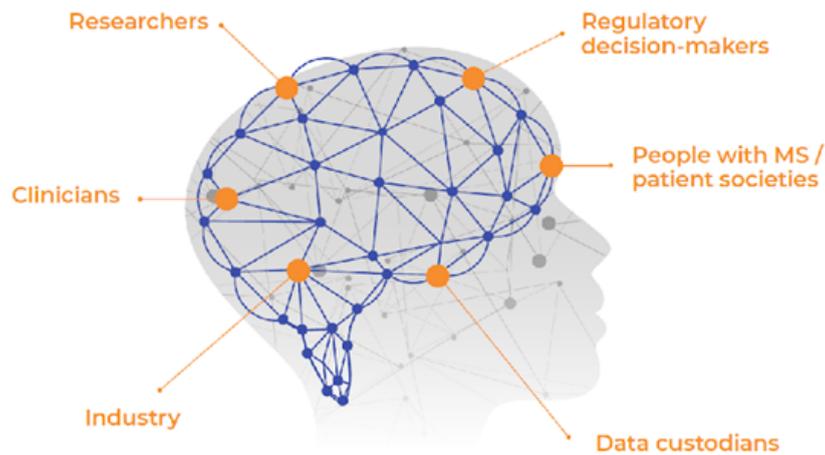


MS DATA ALLIANCE

STAKEHOLDER NEEDS ASSESSMENT

2021

REPORT



Introduction

The Multiple Sclerosis Data Alliance (MSDA) initiative arose from the passionate and successful advocacy work for better use of real-world data (RWD) performed by the European Multiple Sclerosis Platform (EMSP). We believe that RWD can fill in many gaps in our knowledge on MS and thereby transform the care of people with Multiple Sclerosis (MS). Several brainstorming sessions of the MSDA working group during 2018 and 2019 resulted in a concrete **kick-off in November 2019** with the idealistic point of view to raise the voice of people with MS through better and more efficient use of RWD. Since 2020, MSDA has acted as an independent multi-stakeholder initiative under the umbrella of the European Charcot Foundation (ECF) with ECF acting as the legal entity. Six core partners drive the initiative forward: EMSP, ECF, the European Institute For Innovation Through Health Data [i~HD], and the universities UHasselt, KU Leuven and Universitätsmedizin Göttingen. A consortium agreement between these six core partners was signed for a period of 3 years (2020 - 2023).

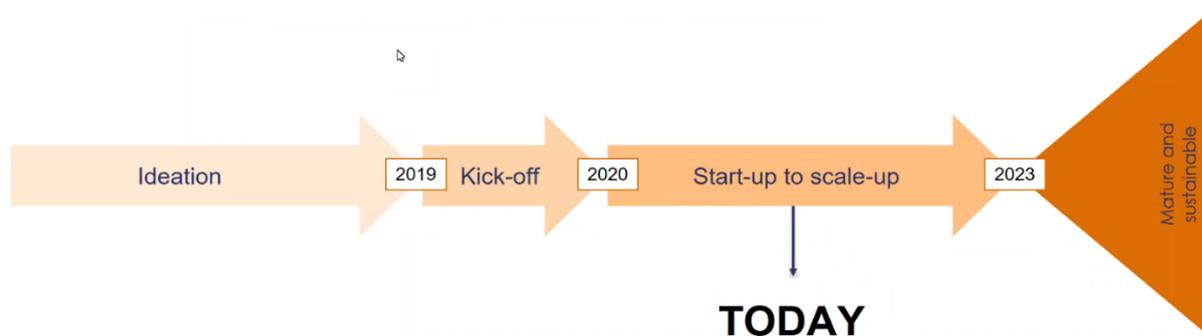


Figure 1: The past, the present and the future of the MSDA

Today, the MSDA reaches an important milestone where it is **ready to evolve** from a kind of start-up organization to a **mature and sustainable multi-stakeholder global data alliance** that (i) is configured and governed in a way that maximizes engagement and trust of the MS community worldwide and (ii) focuses on the right priorities to enable maximum use of MS data to improve knowledge discovery and use to advance the care and the health outcomes of MS patients. The MSDA conducted a **stakeholder needs assessment** in order to better understand what role it should play to support the MS data community, and how to improve its strategy - in co-creation with its stakeholders - in moving forward to a mature and sustainable organization.

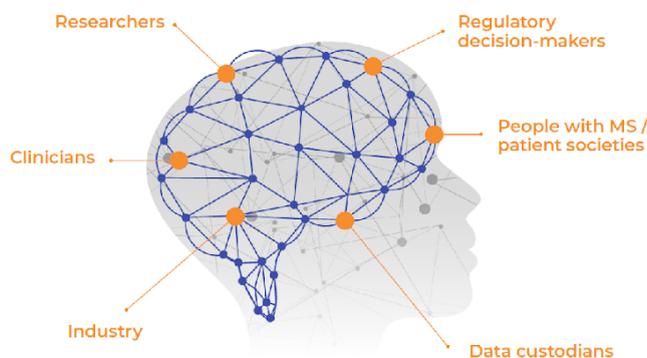


Figure 2: The MSDA has 6 key stakeholders: researchers, clinicians, data custodians, people with MS/ patient societies, industry and regulatory decision makers

We consulted with our stakeholders in **3 stakeholder focus group** meetings in July 2021:

- Stakeholder meeting 1: people with MS/patient societies
- Stakeholder meeting 2: data custodians, academic researchers and clinicians
- Stakeholder meeting 3: Industry and private researchers

The figure below (Figure 3) illustrates the geographical distribution of stakeholder representatives participating in the stakeholder focus group meetings. We restricted the number of people for every stakeholder focus group to a maximum of 15 people. Invitations were sent out using the MSDA network, the network of the core partners as well as the networks of other umbrella organisations (e.g. MS International Federation (MSIF), Russian Committee for Treatment and Research in MS (RUCTRIMS), Latin American Committee for Treatment and Research in MS (LACTRIMS), ...). Participants were selected based on a “first come - first served” basis in combination with ensuring global distribution as much as possible.

We focused on receiving sufficiently broad consultation of our stakeholders on the following main overarching questions:

- What are the **key activities** the MSDA should be focusing on to address the most urgent needs related to scaling-up real-world MS data? ([see Section I](#))
- What are the **key values and governance principles** the MSDA and its members should adhere to co-create a trustworthy, transparent and sustainable real-world MS data ecosystem? ([see Section II](#))

In addition to these stakeholder focus group meetings, an **online survey** was sent out via the EMSP network to collect more information and direct answers from people with MS. A total of 59 respondents have participated in the online survey, including 51 were people with MS, 3 caregivers of people with MS, 4 MS society staff members and 1 physician.

This report summarizes the outcome of this set of virtual stakeholder focus group meetings as well as the key findings of this online survey. This report is used to **inspire and steer further discussions** within the core group and the advisory group to fine-tune the future plans and governance of the MSDA.

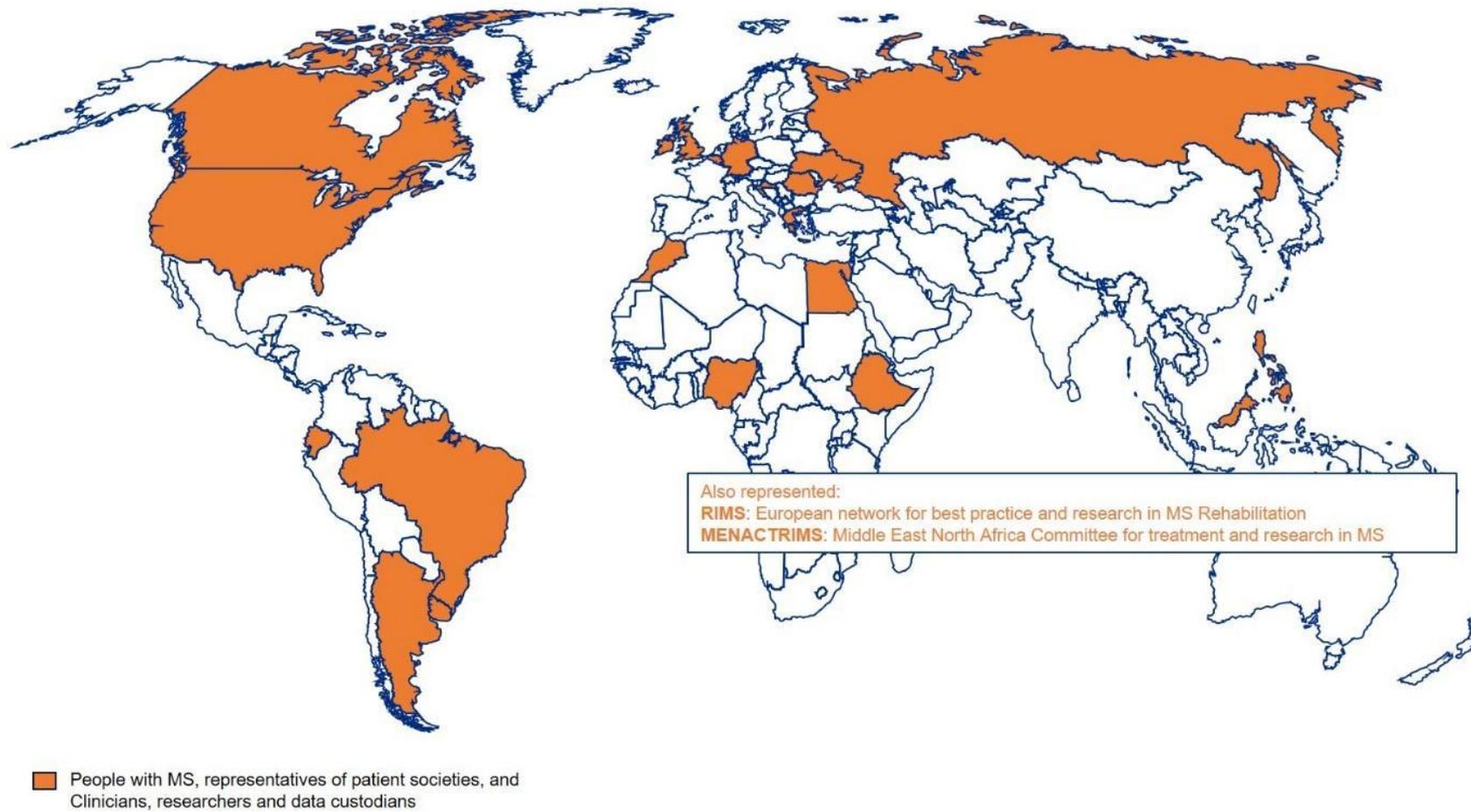


Figure 3: Geographical overview of people with MS, representatives of patient societies, clinicians, researchers and data custodians participating in the stakeholder focus group meetings of July 2021 (stakeholder needs assessments)

Section I: MSDA key activities and priorities

In this section, we focus on the question:

‘What are the key activities the MSDA should be focusing on moving forward to address the most urgent needs related to scaling-up real-world MS data?’

The MSDA aims to overcome the barriers when scaling-up real-world MS data. We want to focus our activities so that we can maximally support the needs of our stakeholders. It became very clear in the stakeholder focus group meetings that the most urgent needs were very different depending on who we were talking to. It was interesting to notice that these differences in needs were not due to the fact that they represented different stakeholders (= the people with MS, MS societies, clinicians, researchers, data custodians and industry). The needs formulated depended heavily on a combination of:

- The level of **awareness of the relevance** of real-world MS data
- The level of **engagement to actively contribute** to the real-world MS data ecosystem
- The level of being **informed about how to contribute** to the real-world MS data ecosystem
- The level of **experience in actively participating** in large scale real-world collaborative initiative

In Figure 4, we pragmatically categorise four levels of stakeholders based on awareness, engagement, contribution and experience on real-world MS data use. We introduce 4 personas (Amina, Jonathan, Zaïd and Ellen) to make these four levels more concrete.

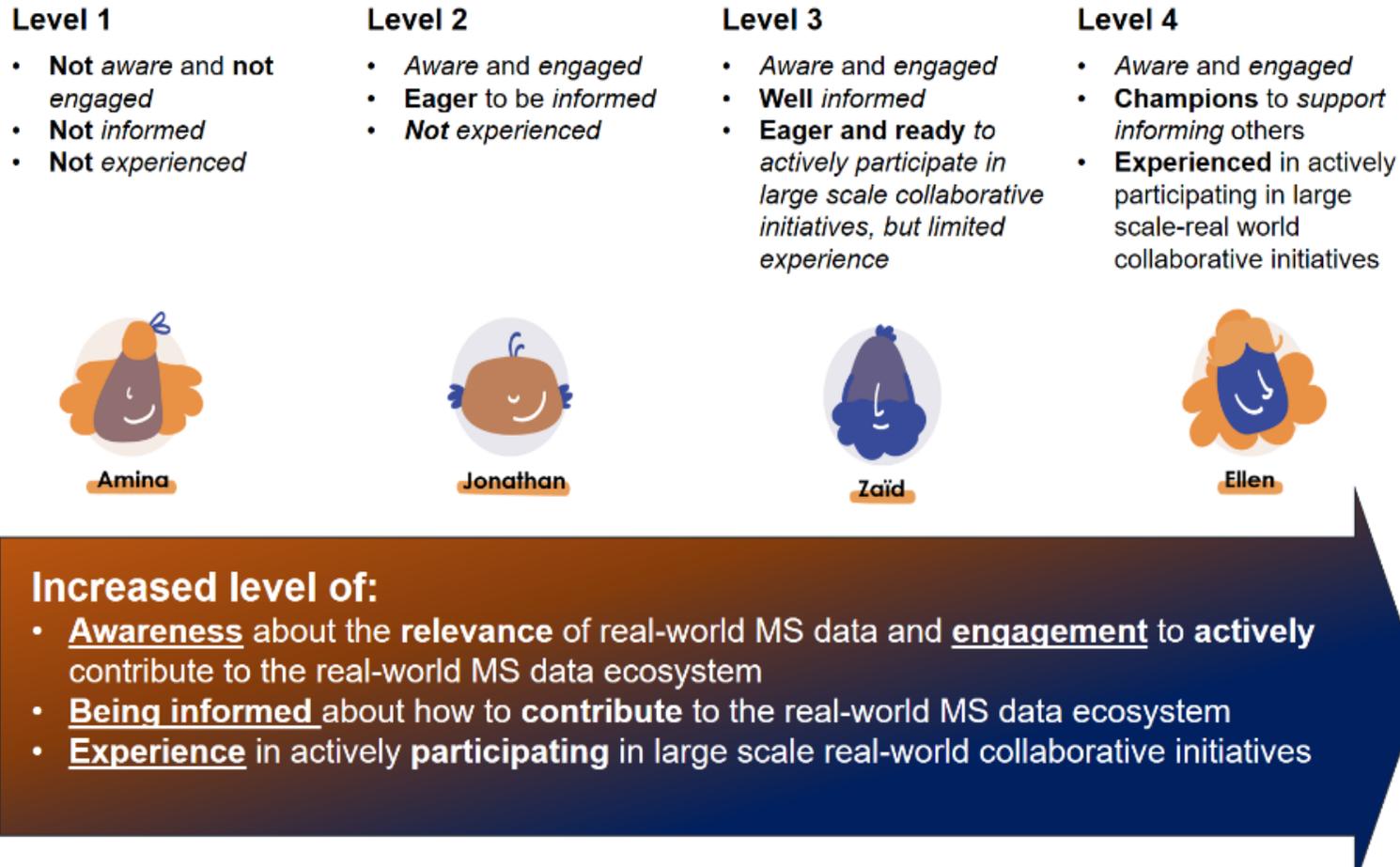


Figure 4: MSDA stakeholders classified into 4 different levels based on awareness, engagement, contribution and experience about real-world MS data use.

What do people like Amina need and how can the MSDA help them?

 <p>Amina</p>	<p>Amina is a clinician*. She is unfamiliar with the term “real-world data”, although she collects a lot of data while she is treating and following-up people with MS in her practice. However, she does not participate within a national registry initiative and is also not motivated to do so. She is unaware why it is useful to systematically collect and share data. She is not engaged in any multi-governance debate that will affect her once the debate is closed (e.g. what variables should be collected in the context of care,...).</p> <p><i>* People like Amina can just as well be a person with MS, a volunteer in an MS society, a researcher, a regulator or an employee of a company.</i></p>
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People like Amina heavily affect the real-world MS data ecosystem. The following examples of quotes formulated during the stakeholder focus group meeting clearly illustrate this:

- *“It is extremely difficult to motivate clinicians in our country to contribute to the national registry initiative. Collecting data is extremely time consuming and clinicians want to focus the time they have on their patients.”*
- *“Compliance to contribute patient-reported data is low. In the beginning, everyone is excited about a “new and fancy” app, but after a few days (sometimes weeks), people with MS lose interest rapidly”. This greatly affects the quality of the data.*
- *“Regulators in our country do not “believe” in real-world data. There is no support whatsoever in setting up our registries. Even worse, when we manage to set-up a registry (on our own), they do not acknowledge the evidence generated.”*
- *“Even during the COVID-19 pandemic, it was very difficult to convince our colleagues to contribute data to the COVID-19 in MS global data sharing initiative.”*

People like Amina are not aware of the existence of the MSDA and *vice versa*, or alternatively, they do not react to invites to participate in stakeholder meetings, nor do they have any interest to be involved in MSDA activities. Therefore, the MSDA will never reach people like Amina directly. However, the MSDA can generate material that can be used by other stakeholders (so called ‘**local ambassadors**’) to inspire Amina and raise awareness on the relevance of collecting and sharing real-world data. To motivate a non-expert audience in particular, it is important to make information understandable and digestible. Some examples of concrete actions the MSDA could do to increase the awareness and engagement of people like Amina with the help of local ambassadors are:

- **Showcase the impact of the reuse of real-world data.** For example, by sharing success stories of collaborative research projects explained for a laymen non-expert audience. This could be done through the MSDA website. It’s clear from the EMSP survey that there is a need to showcase the impact of the use of real-world data since 60% of the respondents to the survey did not know any example of how sharing data helped patients in real-life.
- **Advocate the message “Real-world data - Why should you care?”**, explaining the

relevance from different stakeholder's perspectives. This can be done by for example generating a one-page flyer for every stakeholder separately or by organising and/or hosting local events.

Local ambassadors can use generic MSDA promotional materials (e.g. flyers, videos) and adapt them for **local needs** (translation, local stories, different versions for different audiences). An important remark that was consistently repeated is that materials should ideally be translated into different languages. The use of English does indeed limit the reach for non-English speakers.

What do people like Jonathan need and how can the MSDA help them?

 <p>Jonathan</p>	<p>Jonathan is volunteer in an MS society*. He is inspired by the success stories of powerful real-world MS data initiatives. However, there is currently no registry in his country. He has talked to several key opinion leaders in his country and he managed to convince the MS society as well as several clinicians in his country to initiate a national registry initiative, but he has many questions and is eager to learn how to start this exciting endeavor.</p> <p><i>* People like Jonathan can just as well be a clinician, a volunteer in an MS society, a researcher, a regulator or an employee of a company.</i></p>
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People like Jonathan have many questions. Examples include:

- **How to set-up a registry?** More than half (57%) of the respondents to the survey did not know how health data can be collected. How have other countries done it? What worked well? What didn't? Which software systems for data acquisition already exist? What are the pros and cons of the different systems available?
- **What data should be included?** What variables are crucial to be collected? How should we collect the variables in such a way that we can optimally use the data downstream?
- **How to handle data to ensure ethical and legal requirements?** (e.g. informed consent, General Data Protection Regulation).
- **How should the data be analysed, handled and/or interpreted?**

Several exciting examples of activities to support people like Jonathan were formulated:

- MSDA acts as a **“first-stop-shop”** for questions related to data handling and analyses. The website could serve as an information portal for the most frequently asked questions. In addition to that, easily accessible “helpdesk” services for more specific questions can be provided.
- **Developing or disseminating existing educational resources** on different topics related to data handling and analyses (e.g. videos published on the MSDA website about data harmonization or how the global data sharing initiative (GDSI) was executed). The questions listed above can serve as an inspiration for the most urgent educational material formulated by the stakeholders.
- **Facilitate interaction and collaborations between countries** so that they can learn from each other and maximally leverage knowledge. For example:
 - **Hosting multi-country (online) events**: many people formulated the desire to learn from each other and were in favour of the MSDA to host workshops and/or meetings that bring together different stakeholders regularly to address best practices, tips and tricks, challenges, successes ...
 - **“Twinning” or “buddies”**: Twinning stands for institutional networking. A twinning project strengthens the knowledge in an emerging institution (e.g. a study group interested in setting up a registry). It links the institution with other internationally leading counterparts. Activities can include short term staff exchanges, expert visits, on-site or virtual training, workshops, conference attendance, dissemination and outreach.
- **Connect and build bridges with initiatives outside the MS community** (e.g. other disease areas or more ‘generic initiatives focusing on real-world health data in general)). It is clear that some of the challenges are not specific for MS and we could learn from other activities (e.g. Data Saves Lives, European Health Data and Evidence Network ([EHDEN](#)), ...). The MSDA should connect with these initiatives as much as possible and make sure the MSDA stakeholders are aware of or are involved in these initiatives. A library on the MSDA website cross-referencing to these initiatives is an interesting first step.
- **Formulate guidelines for minimal data collection**: People greatly appreciated the MSDA and MSIF defining the minimal dataset for collecting data on the effect of COVID-19 in MS. This helped to steer data collection very early in the pandemic. Similar guidelines for minimal datasets would be useful (like the Recommended minimal dataset on COVID-19 vaccination that is already published on the [MSDA website](#)).

What do people like Zaïd need and how can the MSDA help them?

 <p>Zaïd</p>	<p>Zaïd is a data custodian*. After years of hard work, he recently managed to set-up a registry in his country and the number of records as well as the data quality is increasing every year. He is very experienced with handling and analysing his own data. He is eager and ready to start collaborating with other similar real-world data initiatives in order to validate, scale and/or compare the insights generated from his data cohort.</p> <p><i>* People like Zaïd can just as well be a person with MS, a clinician, a volunteer in an MS society, a researcher, a regulator or an employee of a company.</i></p>
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People like Zaïd have following needs and questions:

- **How to participate in collaborative research projects?** Examples of topics of interest that were formulated were:
 - Ethical challenges that arise with sharing data
 - Privacy-preservation
 - Data harmonisation
 - Interoperability (e.g. common data models)
 - Data sharing methods and good practices, ...
- **How to increase visibility of their registry and/or cohort?**

The **examples of activities formulated for people like Jonathan are relevant for people like Zaïd as well, so:**

- MSDA could act as a “first-stop-shop” for questions related to data handling and analyses.
- Developing or disseminating existing educational resources on different topics related to data handling and analyses.
- Facilitate interaction and collaborations between countries so that they can learn from each other and maximally leverage knowledge.
- Connect and build bridges with initiatives outside the MS community.
- Formulate guidelines for minimal data collection.

However, for Zaïd these activities require a higher level of detail as well as technical complexity as compared to Jonathan.

Specifically the ongoing **cataloguing activities** of the MSDA have been highlighted as useful for people like Zaïd. Indeed, the MSDA catalogues allow the assessment of metadata of different registries and cohorts. It allows stakeholders a faster understanding of the real-world MS data ecosystem. Next to this, it increases the visibility of emerging registries and cohorts.

What do people like Ellen need and how can the MSDA help them?

 <p>Ellen</p>	<p>Ellen is researcher with a specific interest in MS*. She has an extensive amount of expertise in handling and analysing real-world MS data. She has led large scale collaborative research projects in the past. She has an urgent and crucial question she wants to investigate and is looking for collaborators for this project.</p> <p><i>* People like Ellen can just as well be a person with MS, a clinician, a volunteer in an MS society, a researcher, a data custodian, a regulator or an employee of a company.</i></p>
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The MSDA has showcased during the COVID-19 and MS global data sharing initiative what value it can bring to people like Ellen. Indeed, the MSDA, together with MSIF and many collaborators:

- managed to set-up a global data sharing initiative within a few months, bringing together data from over 80 countries to investigate the effect of COVID-19 in MS.
- delivered the data science expertise required to execute the data handling and analyses pipeline.

We are encouraged to focus on similar activities in the future to support people like Ellen. Other examples of activities that can support people like Ellen are:

- **To reduce the time needed to find relevant MS data sources.** Through the use of the MSDA catalogue, interesting data sources for collaborative research projects can be found. Indeed, the MSDA catalogue currently consists of >40 MS registries and cohorts.
- **Building bridges with other relevant “Big Data” initiatives** within and outside the MS community is, similarly to Jonathan and Zaïd, also useful for people like Ellen. Examples of relevant initiatives the MSDA is currently connected with include the European Health Data and Evidence Network ([EHDEN](#)), observational health data sciences and informatics ([OHDSI](#)), [ELIXIR](#), [eBRAINS](#), ...
- **Increase interoperability between different data sources.** The MSDA should also focus on supporting registries to implement common data models (CDM) that are not MS specific. Examples include [OMOP](#) CDM (Observational Medical Outcomes Partnership), [HL7-FHIR](#), [CDISC](#), [Sentinel](#)... The MSDA is currently involved in a working group within the OHDSI consortium to address the complexities that arise with transforming registry-like data sources to the OMOP CDM.
- **Participate in multi-stakeholder discussions related to real-world data.** There are several important multi-stakeholder discussions addressing the ongoing and evolving health data ecosystem that are not MS-specific. The MSDA should strive towards positive changes in policies to ensure maximal impact from real-world MS data. For example in the past, the MSDA has responded to the European Medicine Agency (EMA) guidelines on registry-based studies and participated in the EMA workshop on a data standards strategy in May 2021.

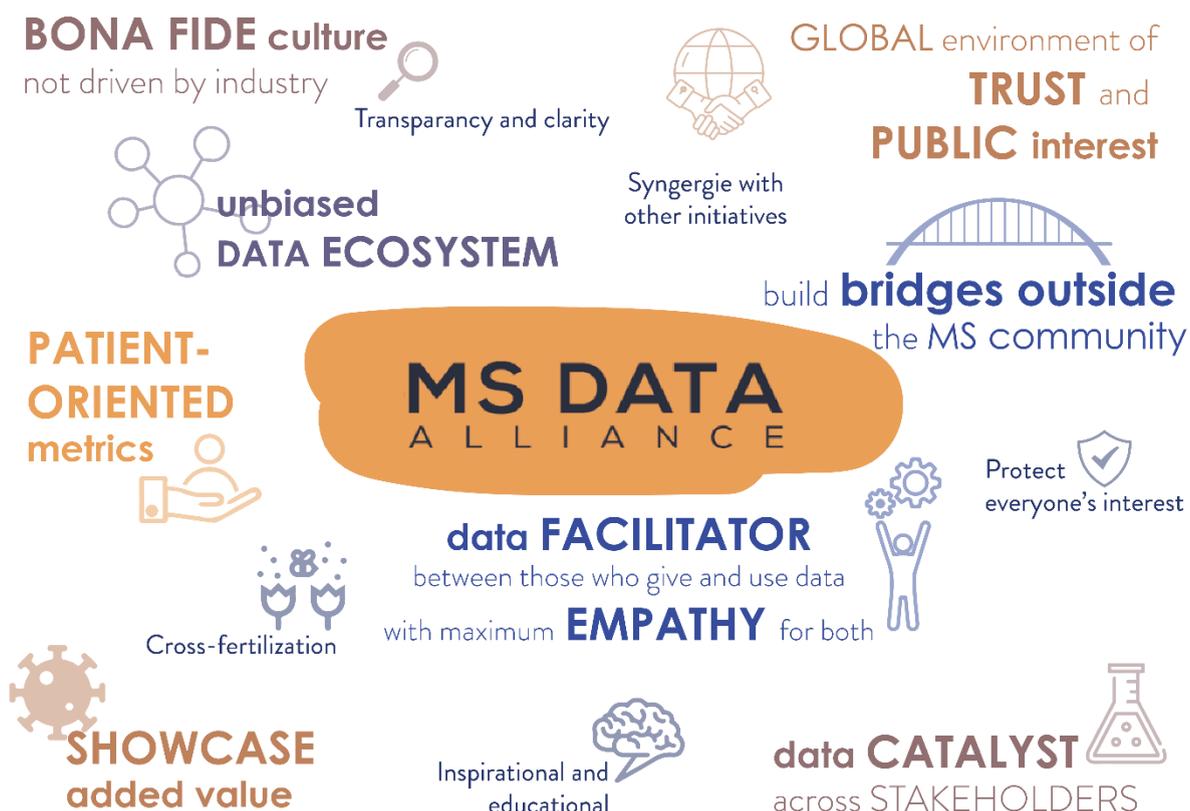
Section II: MSDA key values and governance

principles

To ensure that MSDA evolves into a highly trusted and high-impact research organization, we consulted each SFG on the following topic:

‘What are the key values and governance principles the MSDA and its members should adhere to co-create a trustworthy, transparent and sustainable real-world MS data space?’

During the SFG meetings, the following key words/phrases were repeatedly cited:



Through our series of SFG meetings, we sometimes sensed **different dynamics between stakeholders who collect and/or share data** (people with MS, clinicians and data custodians) and stakeholders who use the data (academics, industry, regulators). Therefore, some suggested values, principles, issues and concerns (= in short '*governance principles*') were mainly raised by people with MS, clinicians and/or data custodians and others were mainly raised by academics, industry representatives and/or regulators. The key challenge of the MSDA is to address all the governance principles to **ensure multi-stakeholder trust**. Indeed, the MSDA should **act as a facilitator** between all stakeholders, with a maximum empathy for all.

- The MSDA should **inspire** and **encourage**, but never judge or oblige. There is no perfect database, and with that in mind, MSDA aims to act as an inspirational and educational organization, formulate recommendations and encourage conversion, **without being judgmental or imposing obligations except those relating to mutual respect**. MSDA should aspire to develop 'gold standards', starting with soft, gentle, nice-to-have standards, until reliable and globally recognized standards are established in the long run.
- The MSDA should always **respect the autonomy of data sources**. For example, the MSDA does not contain any patient-level data or extracts. Indeed, the MSDA catalogue consists only of metadata such as a description of its data set and the kinds of data held in it, and the main permissions and constraints regarding its reuse by external organisations.
- The MSDA activities should always be **in synergy** (and thus not competitive) with other ongoing and emerging MS data initiatives.
- The MSDA should be as "**research-question agnostic**" and inclusive as possible. It should be the aim to facilitate the establishment, findability and use of real-world data within the MS community for all stages of expertise and interest. For example, the MSDA catalogue is not providing any exclusion criteria for e.g. quality metrics since every data source defines it differently.
- The MSDA activities should be relevant at a **global** scale and at the same time **empower local champions**. In doing so, the MSDA should empathize with and support to overcome the current "digital gap" between different regions of the world.
- Although MSDA is sponsored by the industry, it must be at all times clear (e.g. through disclaimers on the website) that **MSDA is a bona fide organization, and that the data is not driven by the industry**. The influence of sponsors within the MSDA must be transparently declared and kept in balance.
- **Transparency is key.**

THANK YOU

The MSDA would like to thank all participants of the stakeholder focus group meetings as well as the people who have shown interest to participate but could unfortunately not participate because the number of participants was limited. We thank Wouter Van Den Bosch, Sofie Ignoul and Kim Pannemans to support us with moderating the sessions. Finally, we would like to thank our sponsors Novartis, Janssen, Merck, Biogen, Roche, Bristol Myers Squibb, Mylan and QMENTA for their continued support.