CML HORIZONS
HYBRID MEETING 2024
Vilnius, Lithuania
24–26 May 2024

Utilising Big Data for CML:

The HARMONY project updates and challenges

Eglys González & Jan Geißler

CML Advocates Network

Silos are for farms, not for pharma!

Courtesy Stefan J. Scherer

Completed CML Studies with Quality of Life Data

Studies	Sponsor	PROs	Sample sizes	Status
SIMPLICITY Fisrt line TKI treatment	Bristol-Myers Squibb	MDASI-CML, FACT-G, CTSQ	1494	Completed
TIPI Phase II	Centre Leon Berard	EORTC QLQ-C30, EORTC-QLQ-CML24	170	Recruiting
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TIGER Phase III	University of Jena	EORTC	717	Completed

Genomic and clinical outcome data of CML patients in different research centers

Country	City	Investigator	Cases committed	Cases transferred	Cases available for analysis
Australia	Adelaide	S. Branford	245	245	245
Canada	Toronto	D. Kim	300	235	-
Czech Republic	Prague	K. Machova-Polakova	220	220	166
Czech Republic	Brno	J Mayer	200	125	-
Germany	Jena	A. Hochhaus, T. Ernst	233	233	233
Italy	Milano	F. Poggi, C. Gambacorti-Passerini	40	19	Being mapped
Kuwait	Kuwait	A. Alhuraiji	40	40	36
Poland		T. Stocklosa	20	pending	
Qatar	Doha	M. Yassin, S. Akiki	100	40	At TTP
Singapore		T. Ong, C. Chuah	57	pending	-
Spain	Gran Canaria	R. Stuckey	112	72	72
Taiwan		L.-Y. Shih	61	61	61
UK	London	J. Apperley	200	pending	-
USA	Oregon	C. Eide	250	pending	-
		Total	**2,078**	**1,290**	**813**

- Big Data can largely influence rapid learning, outcomes, treatments
- Big data can help to understand patterns and prognostic factors that are not visible in small cohorts of patients

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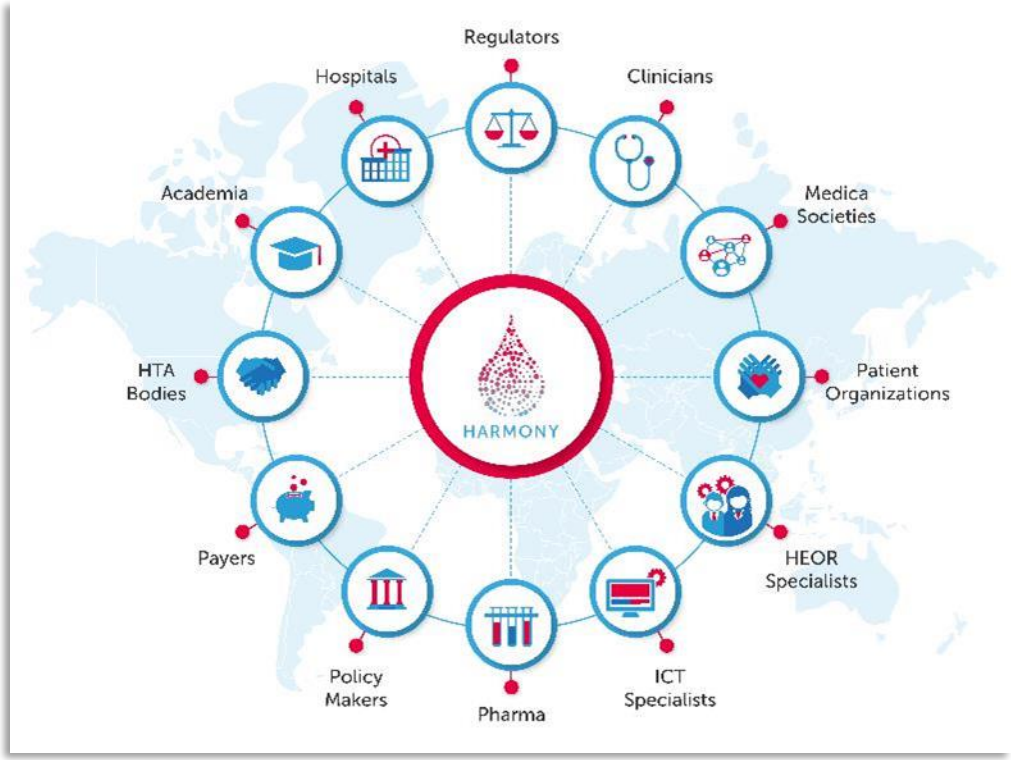
Healthcare Alliance for Resourceful Medicines Offensive against Neoplasms in Hematology



European Public-Private Partnership for big data in hematology

…to collect data and offer big data analytics to speed up research for personalized treatment for blood cancer patients

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Healthcare Alliance for Resourceful Medicines Offensive against Neoplasms in Hematology

HARMONY

January 2017 - June 2023

- ALL — Acute Lymphoblastic Leukemia
- AML — Acute Myeloid Leukemia
- Childhood HMs — Childhood Hematologic Malignancies
- CLL — Chronic Lymphocytic Leukemia
- MM — Multiple Myeloma
- NHL — Non-Hodgkin Lymphoma
- MDS — Myelodysplastic Syndromes

HARMONY PLUS

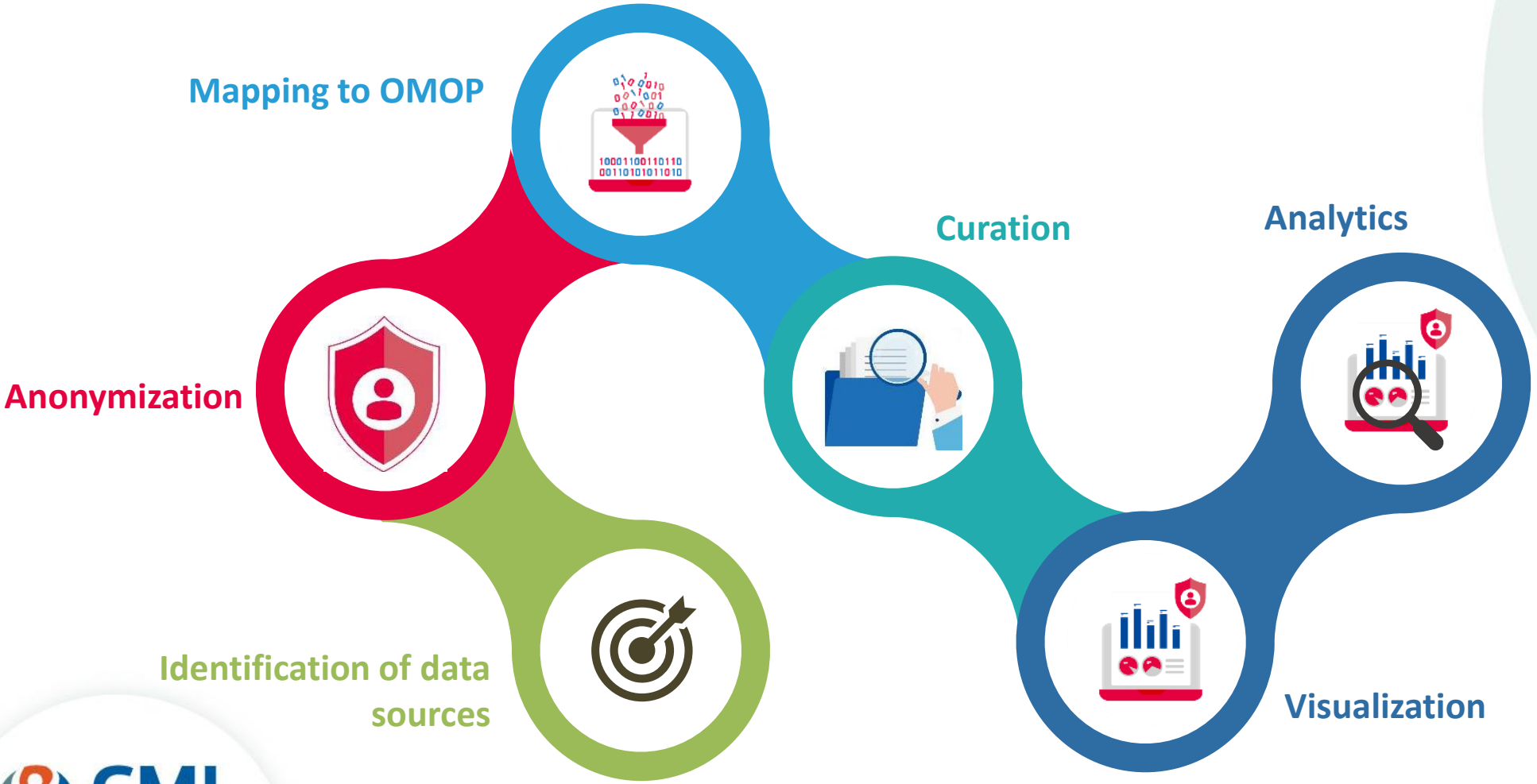
October 2020 – March 2024

- MPN — Myeloproliferative Disorders
- CML — Chronic Myeloid Leukemia
- PV — Polycythaemia Vera
- ET — Essential Thrombocythemia
- Myelo-fibrosis — Myelofibrosis
- HL — Hodgkin's Lymphoma
- WM — Waldenström Macroglobulinaemia
- and other rare blood cancers

HARMONY ALLIANCE FOUNDATION

New legal status in 2024: non-profit research foundation.

HARMONY: How it works



Mapping to OMOP (Observational Medical Outcomes Partnership)

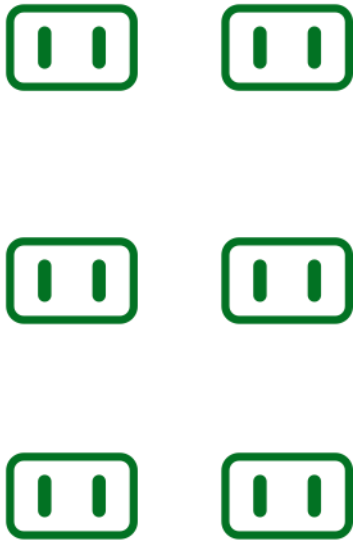
Raw data

North America

Southeast Asia

Europe

OMOPed data



Common

model

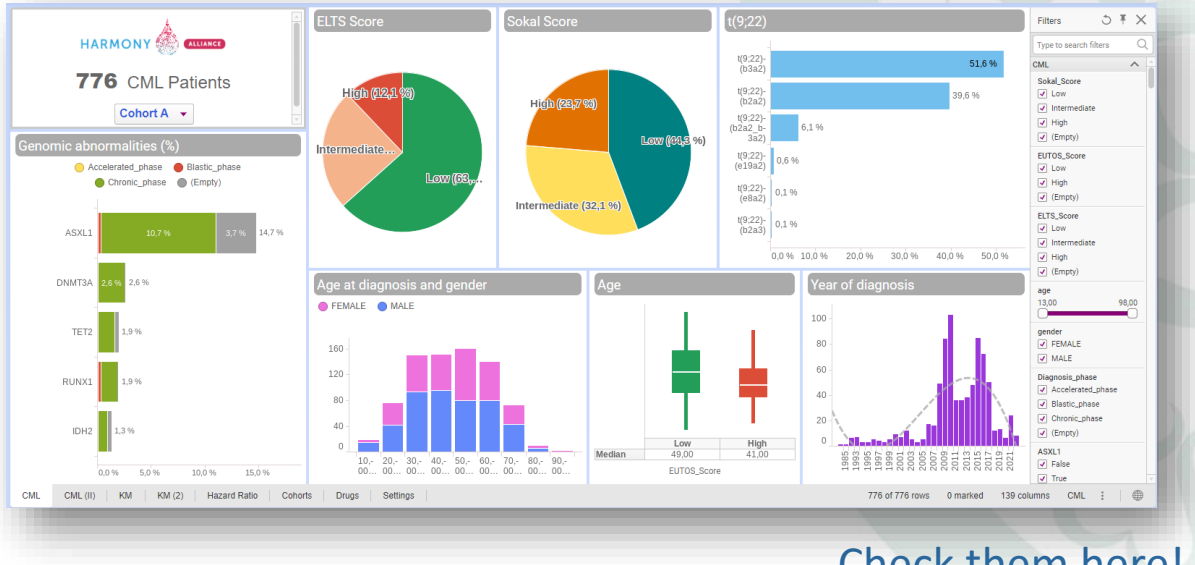
terminologies

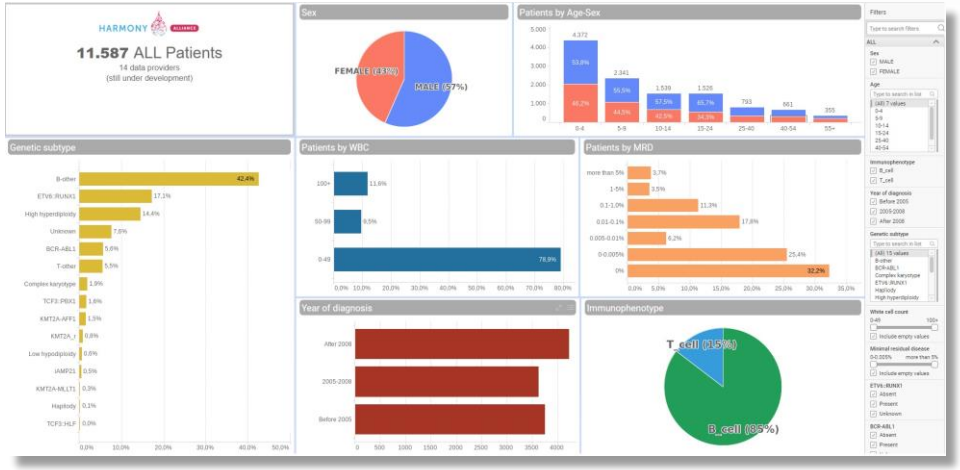
vocabularies

coding schemes

Visualization & Analytics

Data visualization tools available for AML and ALL





Check them here!

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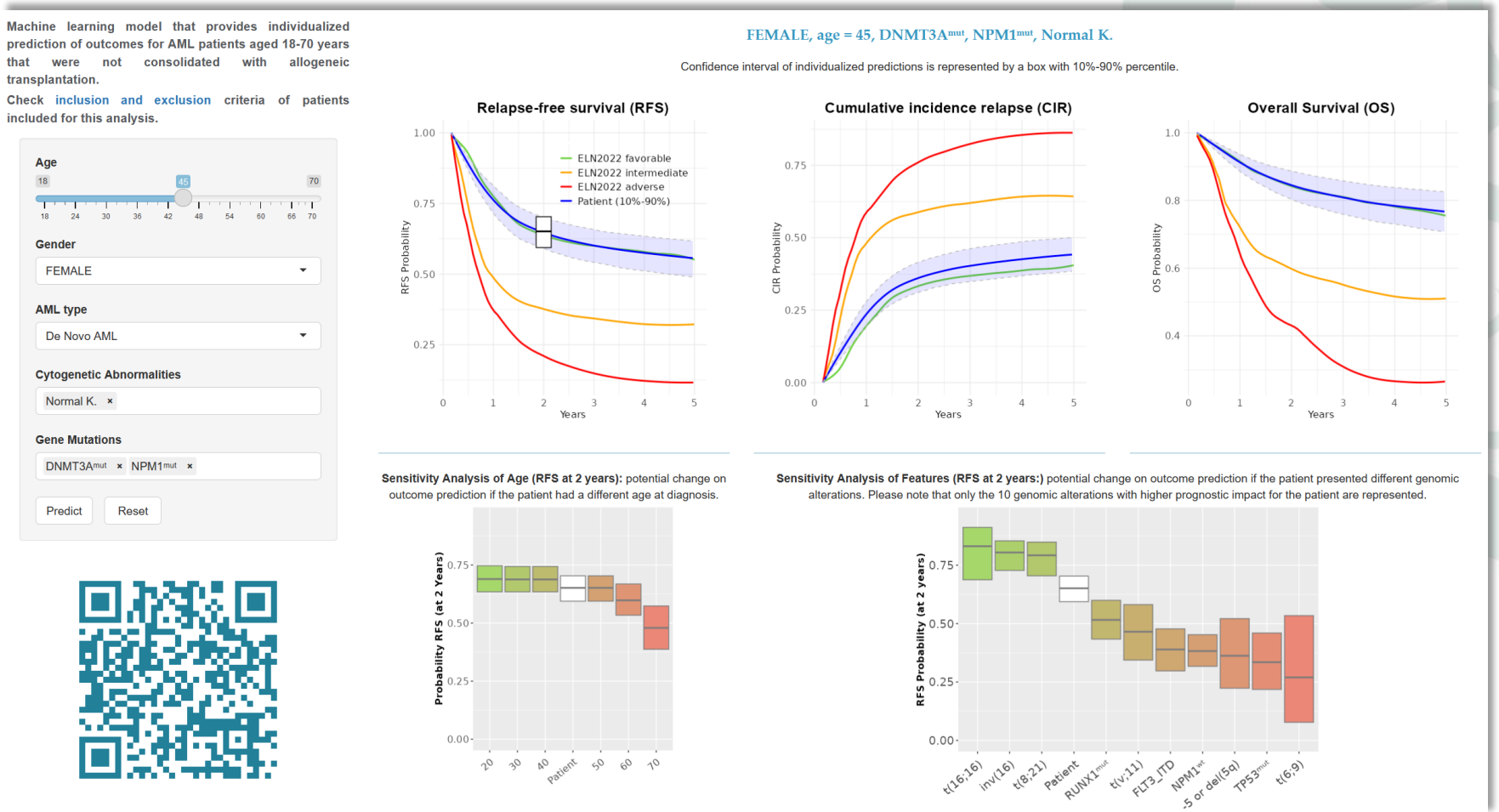
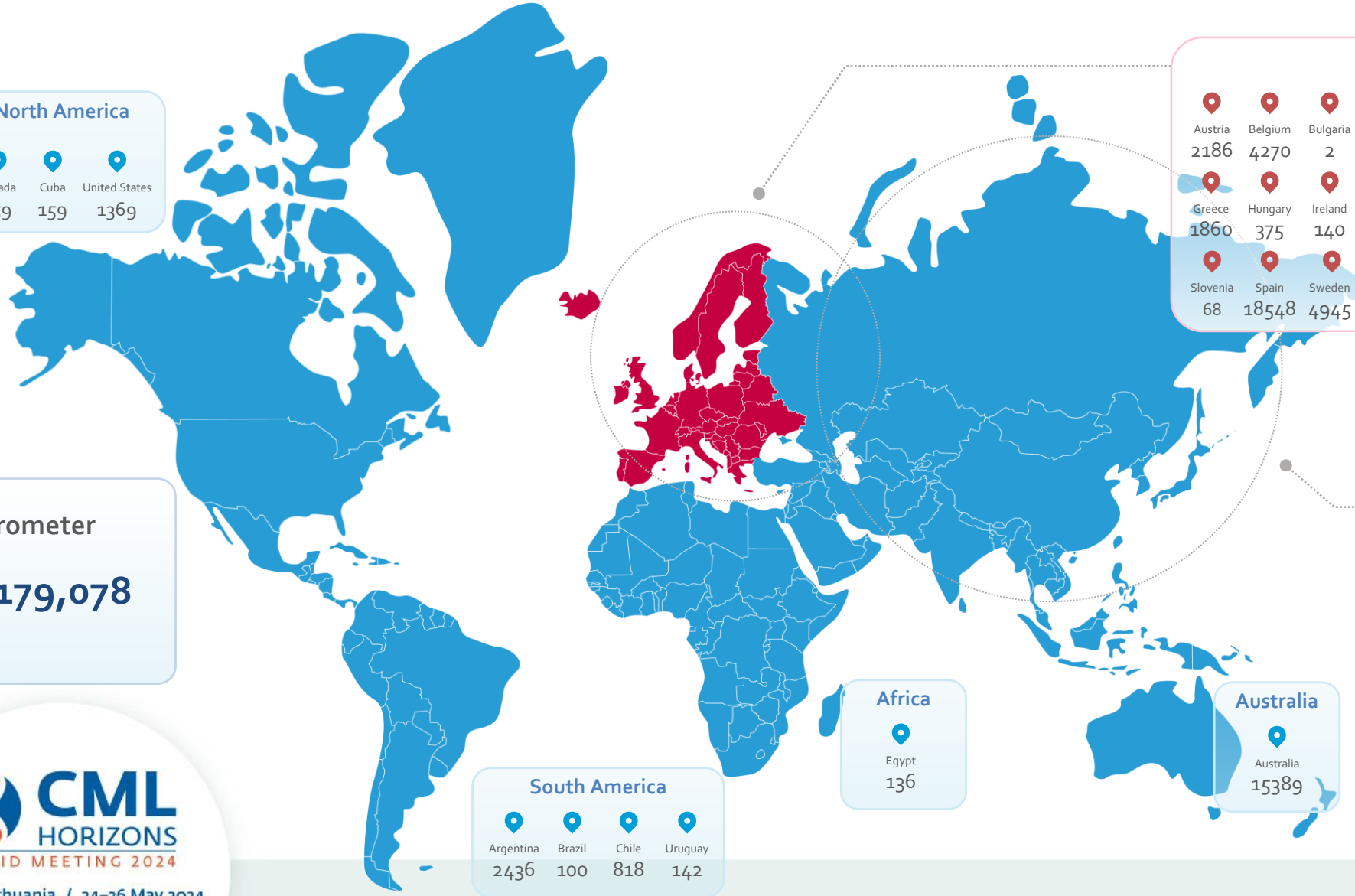
Visualization & Analytics

Individualized prediction of outcomes in AML

…to help **determine in which patient alloHSCT should be performed in CR1**



The HARMONY Data Barometer: cases identified



North America

Canada	Cuba	United States
359	159	1369

Europe

Austria	Belgium	Bulgaria	Croatia	Czech Republic	Denmark	Finland	France	Germany
2186	4270	2	414	11163	1541	9794	7905	28544

Greece	Hungary	Ireland	Italy	Kuwait	Moldova	Poland	Serbia	Slovakia
1860	375	140	19870	319	108	3648	2363	89

Slovenia	Spain	Sweden	Switzerland	The Netherlands	United Kingdom
68	18548	4945	5454	6277	17759

Asia

India	Pakistan	Qatar	Russia
332	64	513	66

Singapore	South Korea	Taiwan
51	52	137

Data Barometer

2024 **179,078**

Africa

Egypt
136

Australia

Australia
15389

International

9313

South America

Argentina	Brazil	Chile	Uruguay
2436	100	818	142

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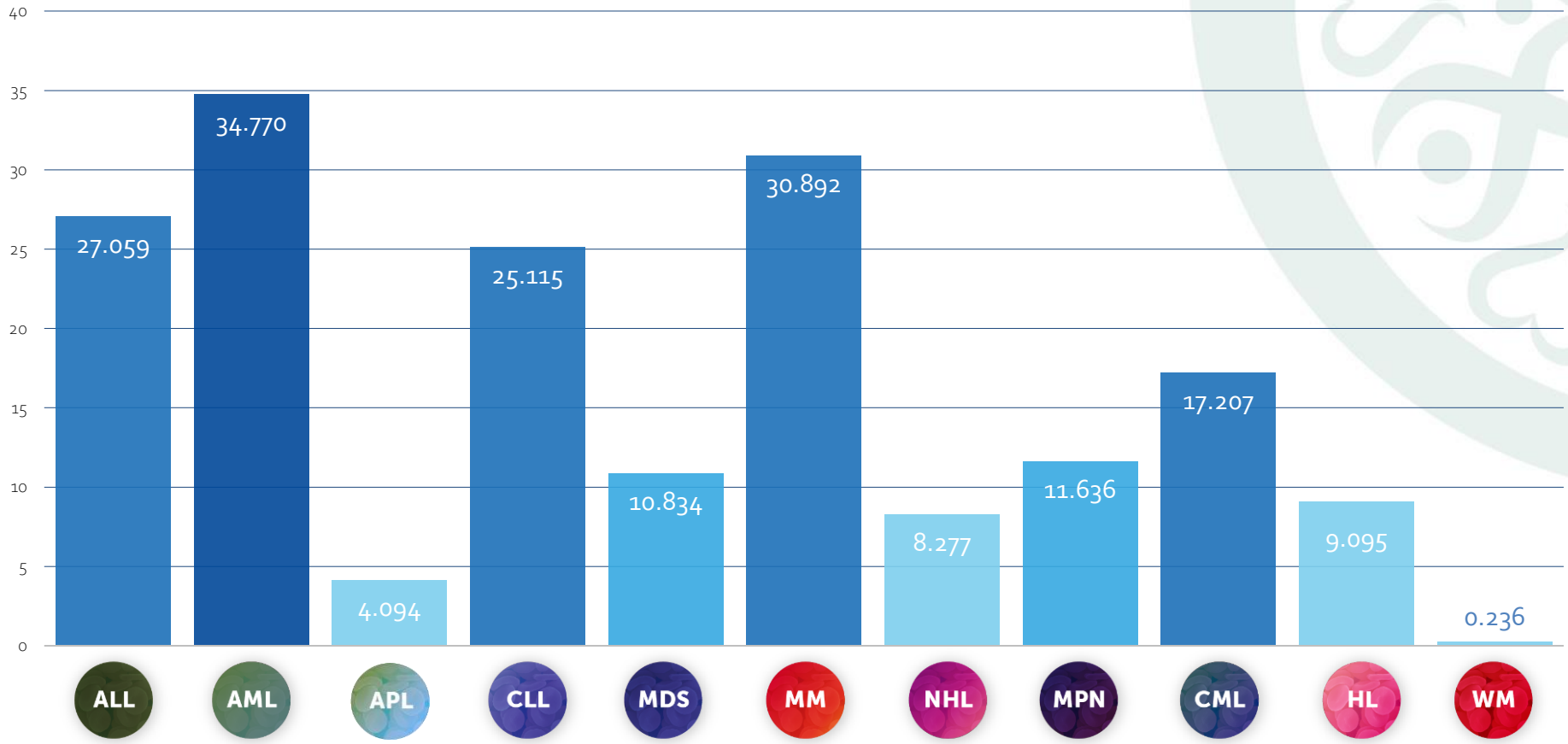
The HARMONY Data Barometer: cases identified

>0K >10K >20K >30K

Data Barometer

2024	179,078
2023	162,000
2022	150,800
2021	97,000
2020	59,500
2019	41,000
2018	34,000
2017	0



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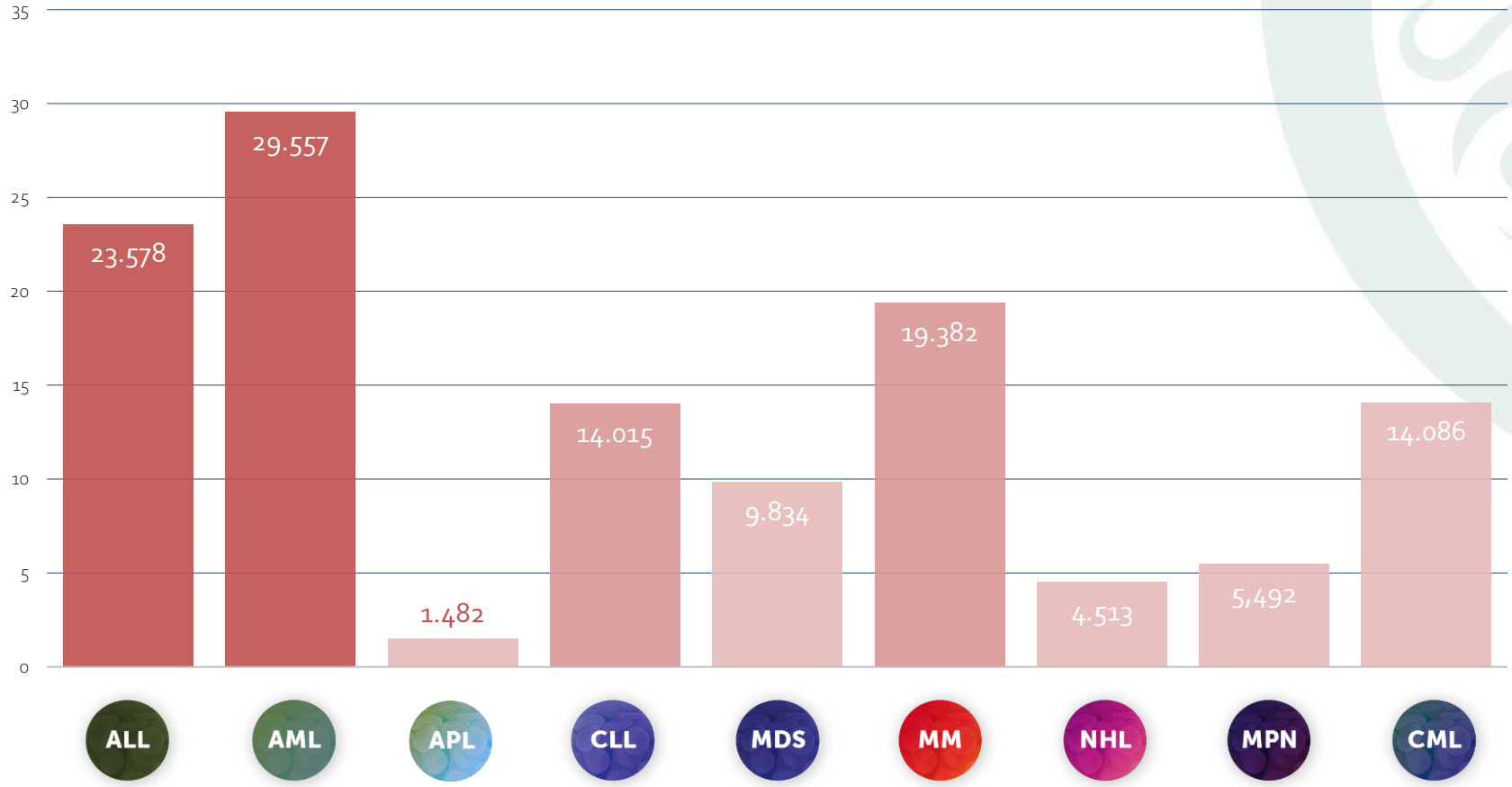
The HARMONY Data Barometer: cases transferred

>0K >10K >20K >30K

Data Barometer

March 2024	121,939
2023	80,004
2022	73,066
2021	61,600
2020	28,000
2019	10,300
2018	1,500
2017	0



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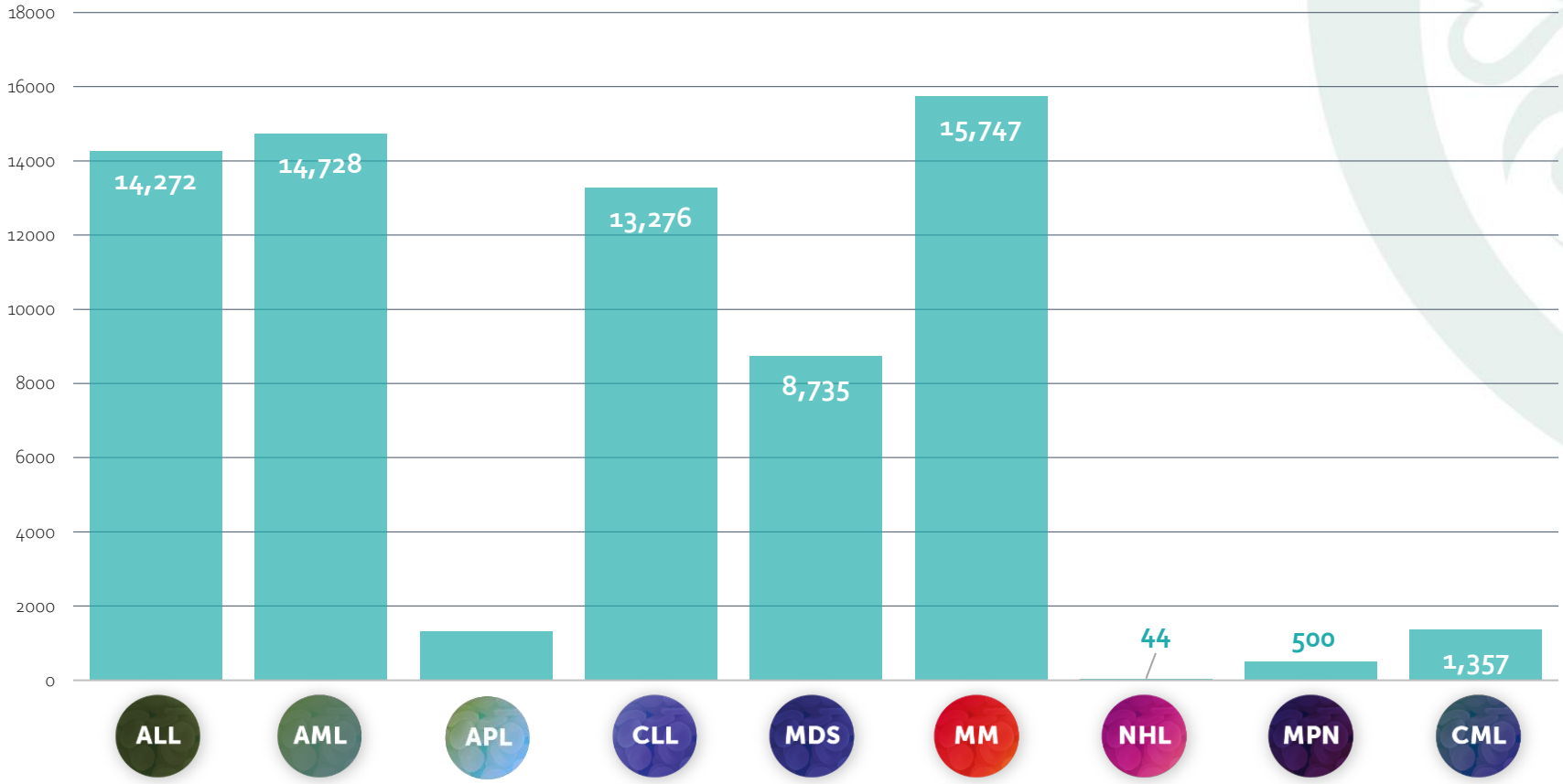
The HARMONY Data Barometer: cases mapped to OMOP

Data Barometer

- March 2024 — 70,000
- 2023 — 47,857
- 2022 — 35,526
- 2021 — 24,618
- 2020 — 18,326
- 2019 — 9,948



HARMONY: What we can do

Consultancy
Research question

current data

new data

Research question

Current research projects

Patients' participation in research projects

WHAT WE HAVE DONE

Patient-led research proposals on CML QoL

1. A comparison study of "toxicity over time" and QoL among all available TKI options

2. Correlation of demographic, disease and therapy factors with toxicity and tolerability of the different TKIs.

3. Prevalence of clinically relevant problems and symptoms in patients with CML

Patient-led research proposals on CML QoL

Studies	Sponsor	PROs	Sample sizes	Status
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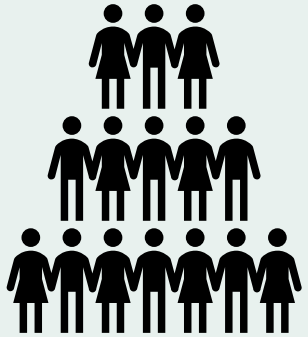
Patient-led research proposals on CML QoL

Organization	Dataset	N cases	PROs
GIMEMA	LEONIDAS (QoL-CML0713)	323	EORTC QLQ-C30, EORTC QLQ-CML24, EORTC QLQ-INFO25 , SCQ
Pfizer	BYOND NCT02228382	163	FACT-Leu
Pfizer	BFORE NCT02130557	536	FACT-Leu
Novartis	ENESTnd	215	FACT-Leu, EQ5D
University of Heidelberg - Mannheim	EURO-SKI	868	EORTC
GIMEMA	GIMEMA QOL-CML0208 GIMEMA QOL-CML0310 GIMEMA CML0811 GIMEMA QOL-CML0916	422 655 130 782	SF 36, PGWB-S, FACIT-Fatigue, MSPSS EORTC

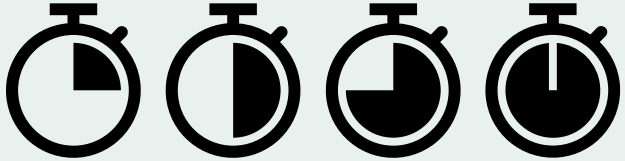
Challenges

Challenges

Similar population according to the research question



Time points data had to be collected



For QoL analysis same questionnaires are required

EORTC QLQ-C30
EORTC QLQ-CML24
EORTC QLQ-INFO25
FACT-Leu
EQ5D
MDASI...

Patient-led research proposals on CML QoL

Status

Conclusions

- ✓ HARMONY represents a strong platform to enable Big Data and real-world data, with already well-established processes
- ✓ Can be fundamental to increase research in connecting clinical outcomes with patient-reported outcomes to help better understand the impact of therapies on daily patient reality
- ✓ Patient participation in big data initiatives is required, increasing roles in data sharing and governance discussions
- ✓ More research questions should come from the patient community

THANK YOU!

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Jan Geißler | jan@cmladvocates.net

BACKUP SLIDES

Role of the patients

LeukaNET
Leukemia Advocates Network

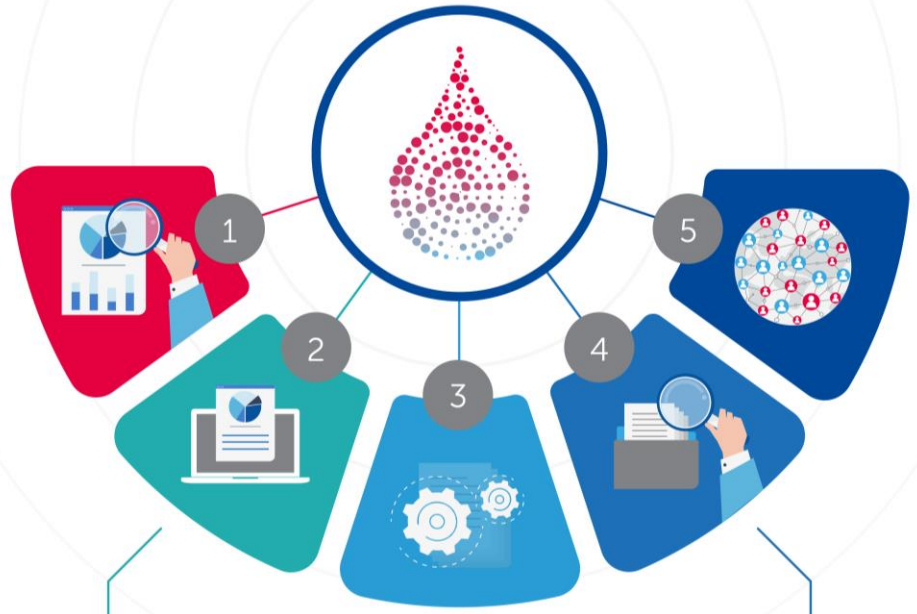
Work Package 4 Leader

WP4 to involve stakeholders in HARMONY, also ensuring POs participated to inform researchers, clinicians, pharmaceutical companies and regulators about patients' needs, concerns, and priorities.

Role of the patients - contributions

- ✓ Development of an Standard Operating Procedure (SOP) to ensure patients' consultation in research projects
- ✓ Contribution in the Delphi process of the definition of Core Outcome Sets (COS) for Hematologic Malignancies
- ✓ Submission of patient-led research proposals that address questions coming from the patient community

Patient involvement in HARMONY



RESEARCH IDEA + DEVELOPMENT OF PROJECT PROPOSAL

Interaction with the HARMONY Patient Cluster

SUBMISSION TO HARMONY

PROJECT IMPLEMENTATION

Interaction with the HARMONY Patient Cluster

ANALYSIS OF RESULTS

Interaction with the HARMONY Patient Cluster

DISSEMINATION OF RESULTS

Interaction with the HARMONY Patient Cluster

Research proposals on CML QoL

1. **A comparison study of "toxicity over time" and QoL among all available TKI options**

Despite TKIs have been widely used for more than two decades, there is still little knowledge on time points, transience, and cumulative effects of all available TKIs. This research project will analyse toxicity and QoL data over time from patients treated with TKIs considering all stages of the disease and variable therapy doses.

Research proposals on CML QoL

2. **Correlation of demographic, disease and therapy factors with toxicity and tolerability of the different TKIs.**

There is still no accurate system to predict individual intolerance to TKIs. This would be of high relevance in improving prognostic factors on therapy-related morbidity, tolerability, and QoL. This research will identify significant correlations between demographic, disease and/or therapy-related conditions with toxicity (CTCAE), tolerability and QoL among treated patients.

Research proposals on CML QoL

3. **Prevalence of clinically relevant problems and symptoms in patients with CML**

Describe prevalence of clinically relevant problems and symptoms in patients with CML in first-line treatment. Analyses will be performed in the overall population and by other key patients' characteristics and type of treatment.

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