



CML Advocates Network

Patients-Generated Data

A practical example: The CML Adherence Study

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Why do patients organizations need to produce data?

- **To prove a point important to patients** that no one else is investigating.
- **To better understand challenges** that patients face and find the solutions to solve or improve them.
- **To improve the drug development process** incorporating (data on) patients' needs
- To prepare data on claims and justifications for **drug reimbursement processes like HTA.**

Why patient-generated evidence?

Example: Adherence of CML patients.

Problem

- Prior studies demonstrated non-adherence is key problem in CML (ADAGIO 2008, Hammersmith)
- Clinical impact proven (Bazeos 2009)
- “This is cancer! Of course MY patients take their medicines!”

Objectives

- Understand patient behaviours associated with adherence
- Help identify the ‘true’ issues behind non-adherence
- Explore cultural influence
- Support development of physician and patient tools to improve patient outcomes

Process



Why patient-generated evidence? Example –Adherence of CML Patients.

1

Pilot study



2

Scientific study



3

Publication

2011-2012

Pilot study to test hypothesis and questionnaire

Build workgroup (8 advocates, 3 experts)

Low effort, little budget

10 countries, 150 patients, recruited via members

Published in

JOURNAL FOR
PATIENT COMPLIANCE
Strategies to enhance Adherence and Health Outcomes
PEER REVIEWED

2013

Scientific study with validated tools

Multi-sponsored project supported by logistics agency

79 countries, 2549 patients, recruited online and offline in 4 months

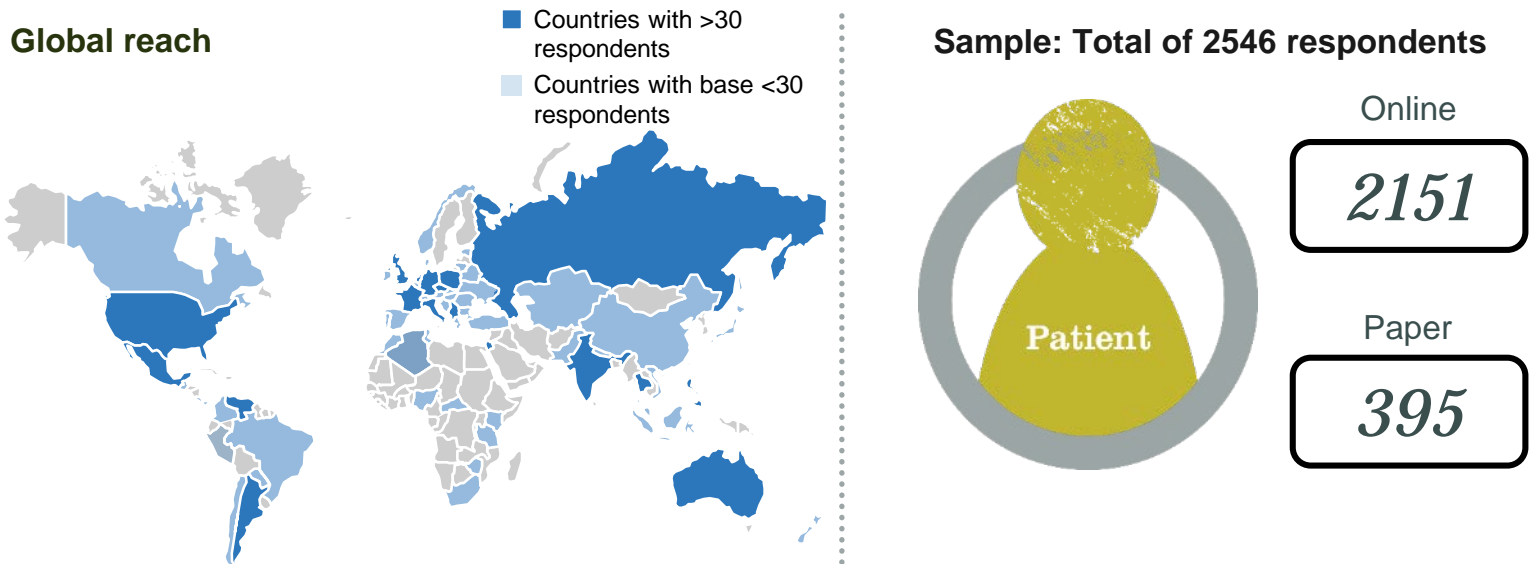
2013-2016

Presented at EHA and ASH in 2013

Publication to be submitted to Blood in 2016



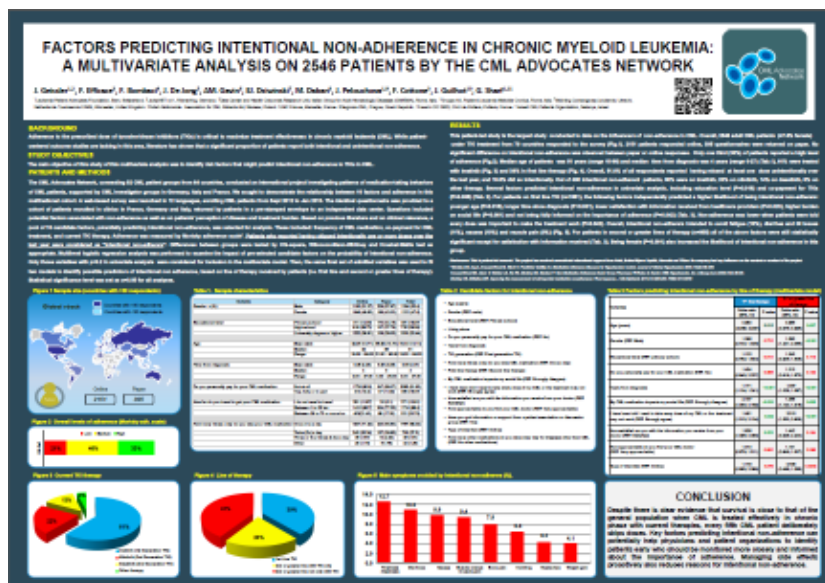
Patient-led adherence research in CML: 12 languages, 2.546 patients, 79 countries, validated tool



Methodology

- **Online** - Recruited by patient associations online & via other methods
- **The Power of a global network**- additional translations(Slovenia, Lebanon, Thailand etc)
- **Paper & Pen** (France, Germany, Italy)
 - recruited by physicians at consultations -- important to minimize bias of online users.
- **Collaboration** with CML experts on steering committee + psychologist + CML study groups
- **Contract with logistics agency** to execute the research, collect data and do analysis.

Patient-led adherence research in CML: 12 languages, 2546 patients, 79 countries, validated tool



**Low adherent:
21%**



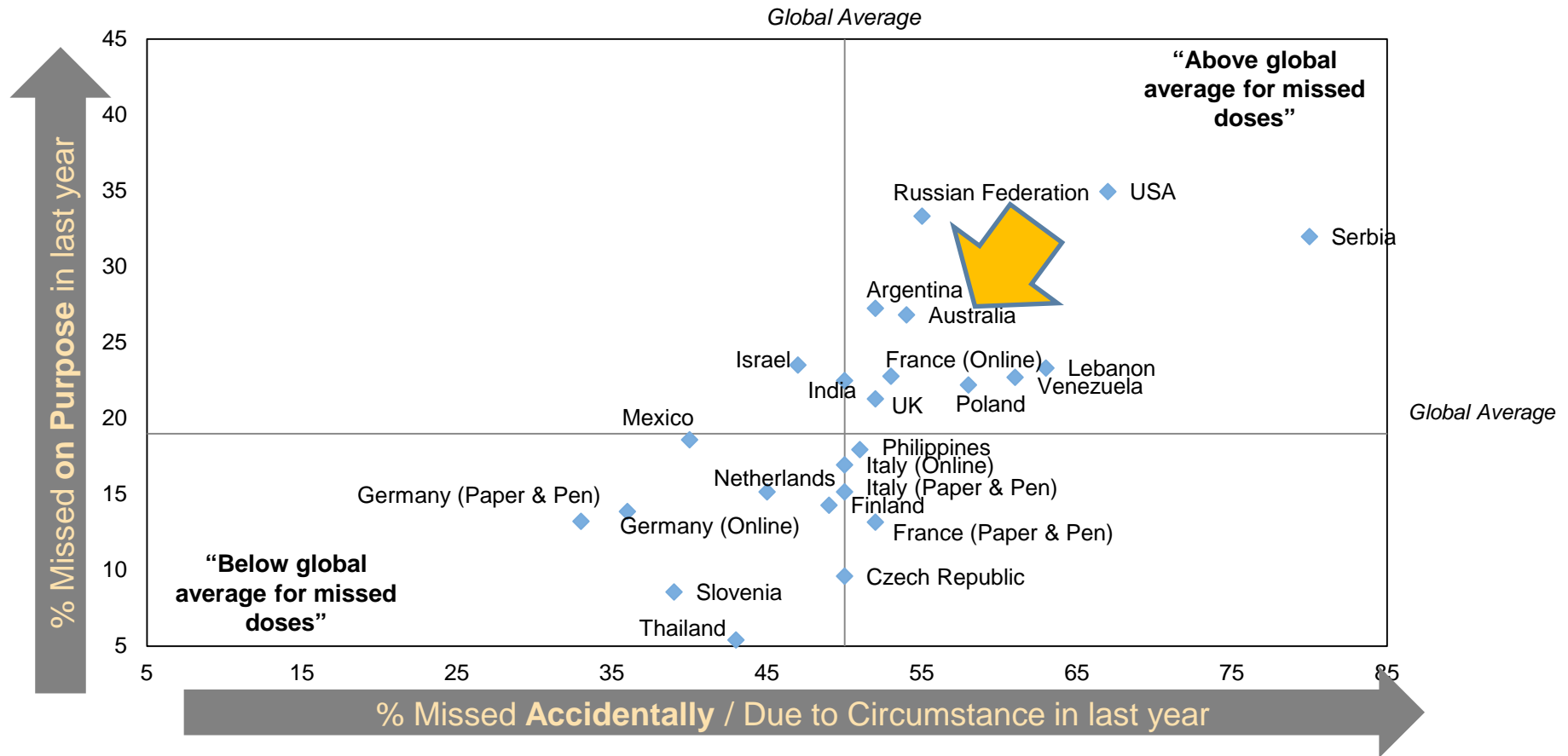
**Medium
adherent: 47%**



**Highly adherent:
33%**



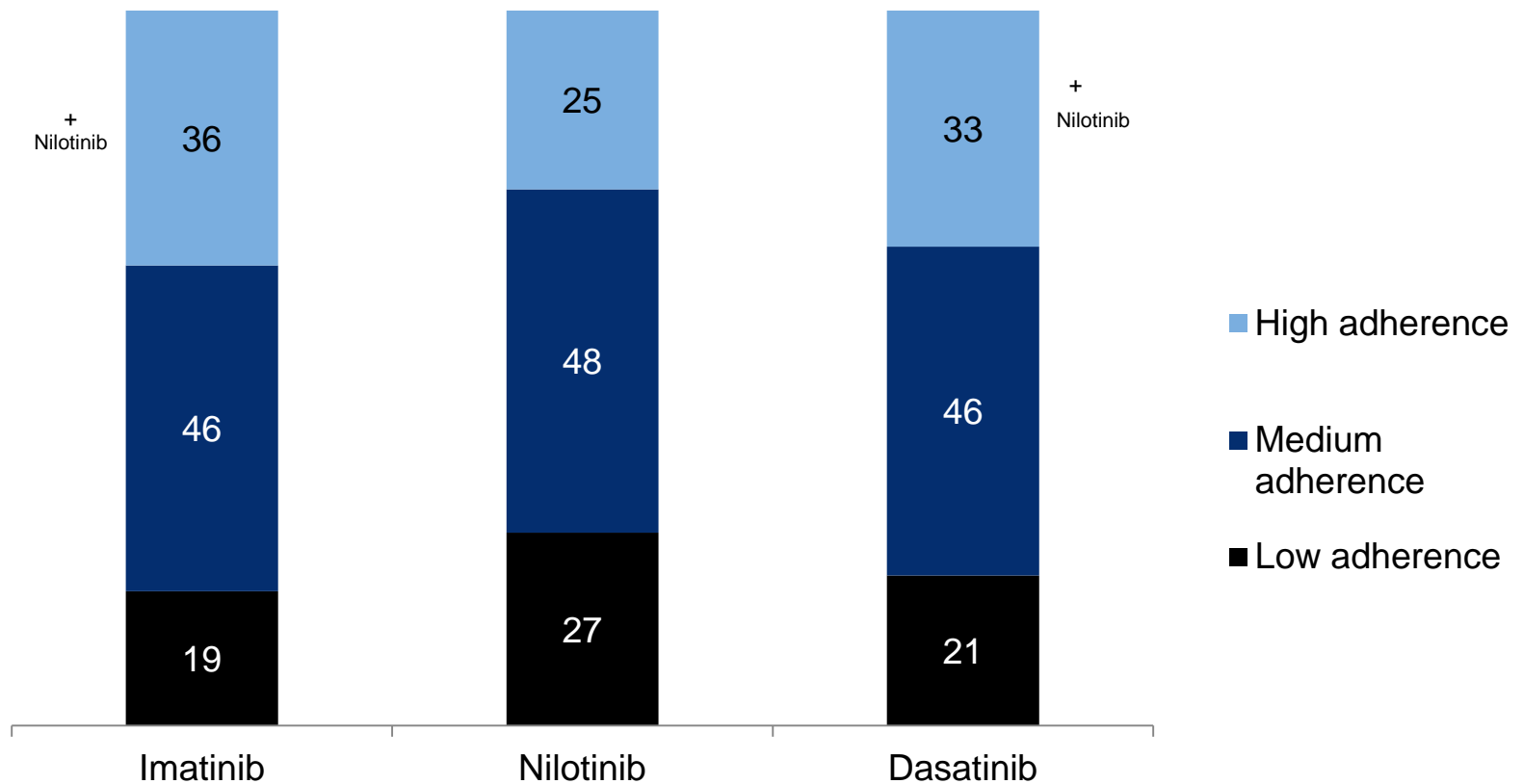
Can a single-country study provide global answers? Certainly not → the power of global patient network



C2a / base=all respondents (n=2546) - Patients sometimes are not able to take their medication as prescribed. In the last month, have you missed a dose accidentally or due to circumstances that were outside of your control? C2c / base=all respondents (n=2546) - In the last year, have you missed a dose accidentally or due to circumstances that were outside of your control? C4a / base=all respondents (n=2546) - Patients sometimes make a conscious decision to miss a dose of medication. In the last month, have you decided to miss a dose? C4b / n=2258 - In the last year, have you decided to miss a dose?

Only patients-driven research can investigate adherence according to specific drugs! Difference between the Pilot study and the full study

Current medication vs. levels of adherence %



Only we PO will be able to prove that adherence is a joint responsibility and is strongly influenced by doctor-patient relationship

High-adherence group is more likely to **discuss missing a dose with their physician**

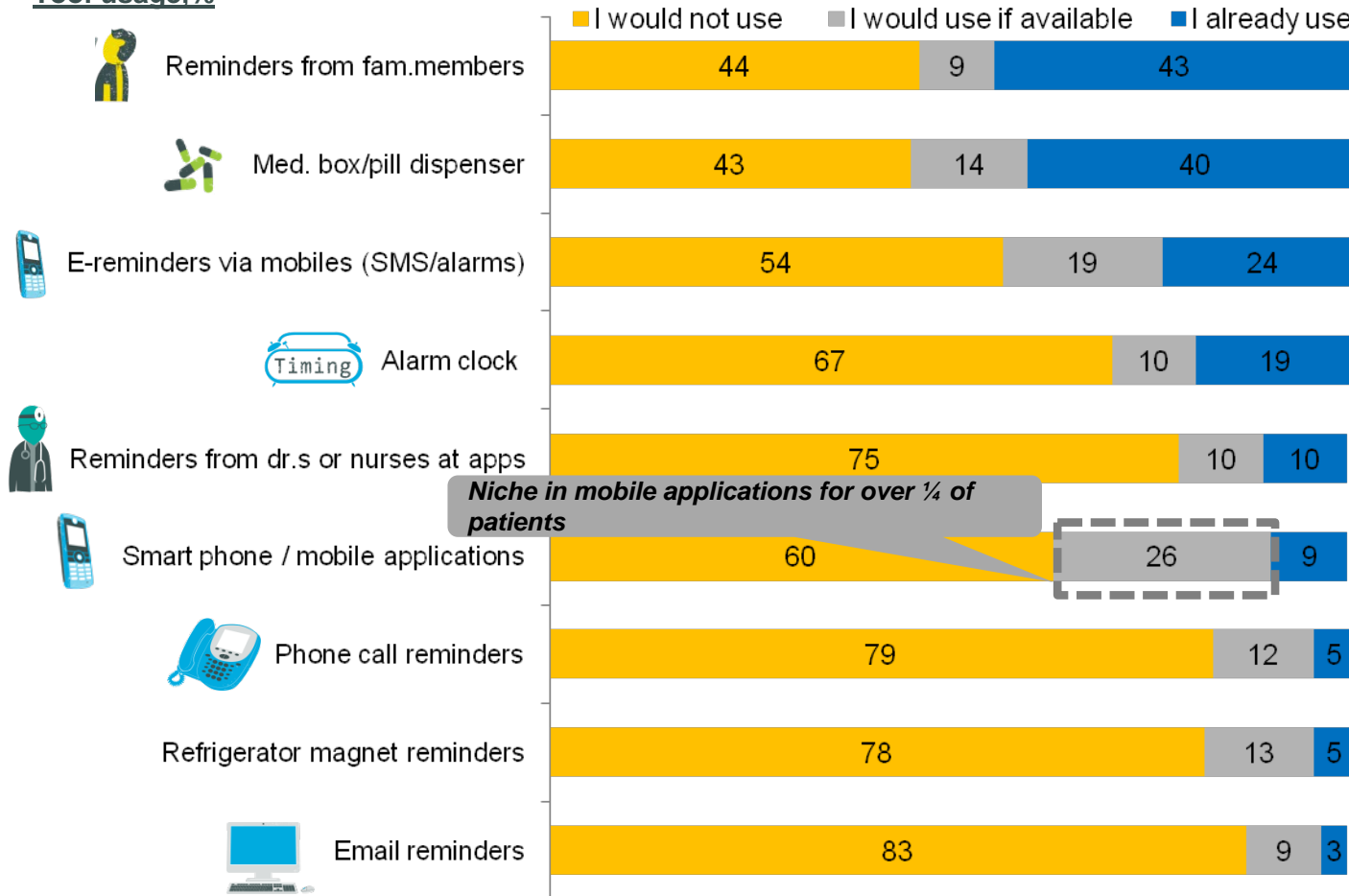
Accidentally	HIGH	LOW	Intentionally	HIGH	LOW
“Yes always”	83%	47%	“Yes always”	85%	48%

High-adherence group is more satisfied with **info received from HCPs, and HCPs are perceived to be approachable**

Info received	HIGH	LOW	HCP is approachable	HIGH	LOW
“Very satisfied”	69%	45%	“Very approachable”	73%	53%
“Not satisfied at all”/ “Somewhat dissatisfied”	4%	16%	“Not very approachable”	7%	14%

Reminders from Family Members, Pill Dispensers Are Primary Tools Used, Followed by Electronic Reminders

Tool usage, %

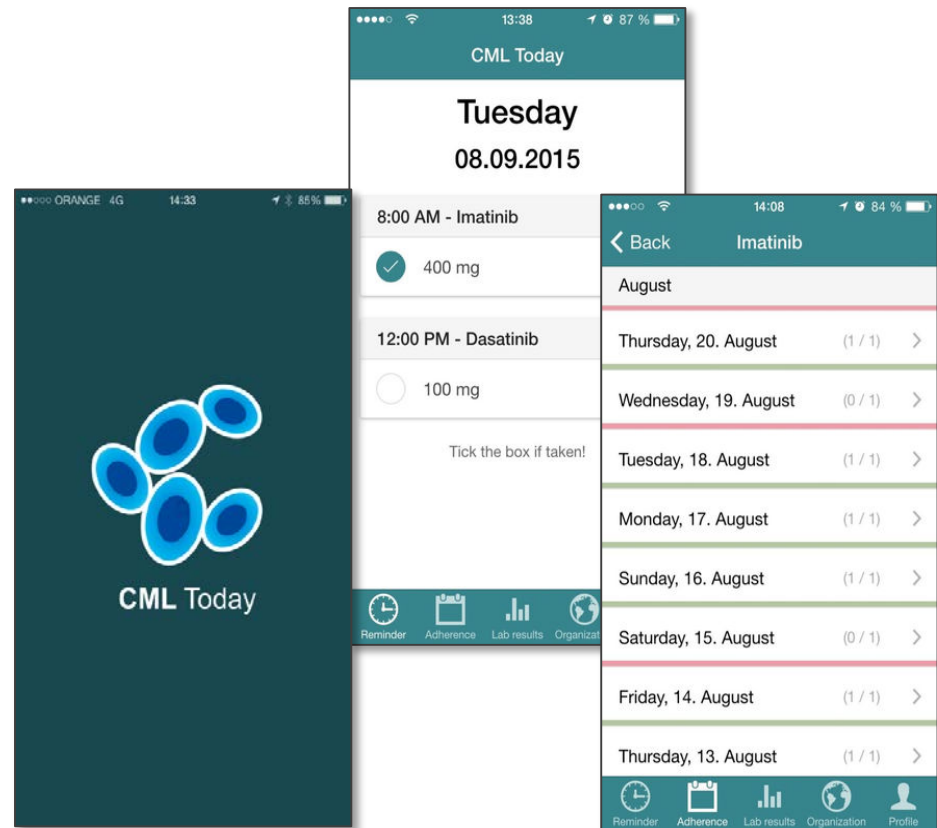


E2 base = all respondents (n = 2546) – Below is a list of tools that could help you to remember to take your CML medication. For each item, please indicate whether you already use such a tool, or if you would use the tool if it were available

Our Mobile App “CML Today”: Tracking adherence and lab results

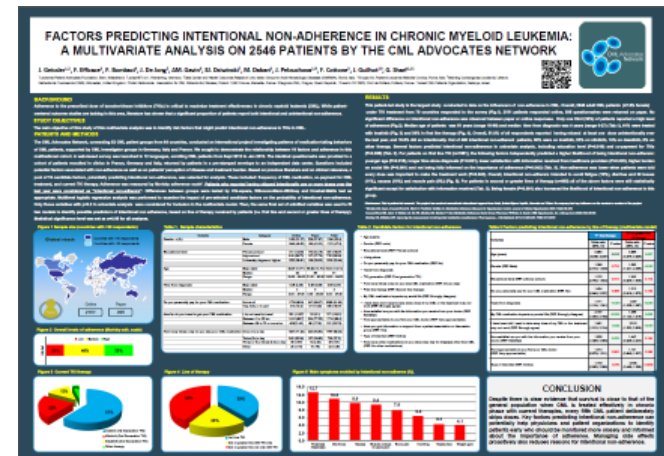


- Track intake of CML medicines to improve adherence
- Track PCR and other lab results
- Additional patient information provided by CML organizations, in local language
- App available in English, French, Spanish, German, Hebrew, Portuguese, Arabic
- Very user-friendly
- Free on iPhone and Android



Successes

- First patient group ever to present data in **oral scientific sessions at European Hematology Association (EHA) congress 2013**. Focus: overall data
- **Poster presented at American Society of Hematology Meeting (ASH) in 2013**. Focus: Risk factors of patients on intentional non-adherence
- **Mobile iPhone/Android App** launched in 2015
- **Publication** just about to be submitted to “Blood” Journal



Lessons learned

- **Be ready** to have enough time and resources allocated
- **Run a pilot** to test your hypothesis and survey/research design
- **Anticipate potential criticism!**
Generate solid data e.g. by using validated tools.
- **Use a reliable agency and medical writers**
(costs \$\$, but worth it) to support collection, run first analysis, write the papers – volunteering is cheaper but takes ages!
- **Publish the data to get acceptance in HCPs!**
(And get reliable help for this)



Thank You

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