



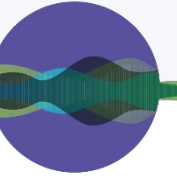
**The agency perspective-  
Patient authorship and plain  
language summaries**

Phil Matthews, PhD, CMPP  
Portfolio Director & Team Lead,  
Envision Pharma Group

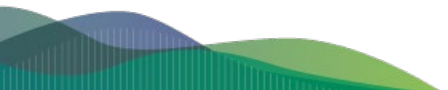
December 10, 2019

# Objectives

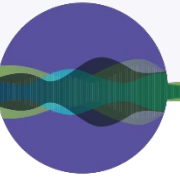
---



- To gain an overview of where patients can be involved in the publications lifecycle
- To explore best practices and real-world examples of patient involvement in 2 key areas:
  - Patient authorship of publications
  - Plain language summaries (PLS)
    - Practical considerations

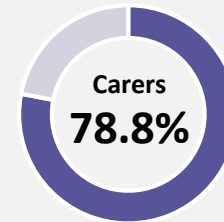
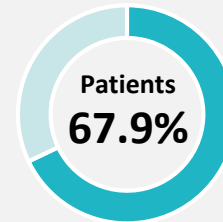


# Many patients want to understand published research: but it isn't always that easy

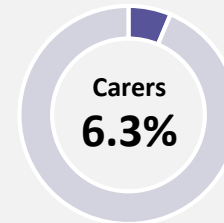
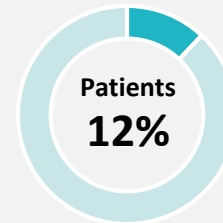


## IN A SURVEY OF PEOPLE WITH FRIEDREICH'S ATAXIA AND THEIR CARERS: \*

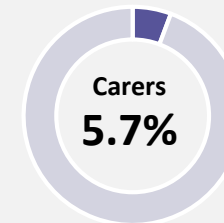
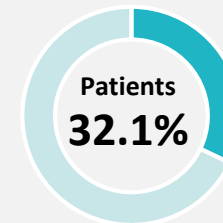
Most patients and carers  
were interested in  
scientific publications  
related to their condition



Few could understand  
scientific publications



Few considered the Internet  
(Facebook, discussion forums,  
etc) to be a useful source for  
better understanding



\*Amelot V, et al. *Pharm Med.* 2017;21:329-37 (study included 28 patients and 35 parents of patients).

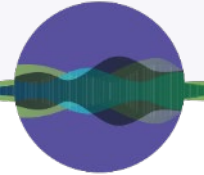
# How do we do it?



“Stakeholders agree that **more effective patient involvement is needed** to ensure that patient needs and priorities are identified and met.

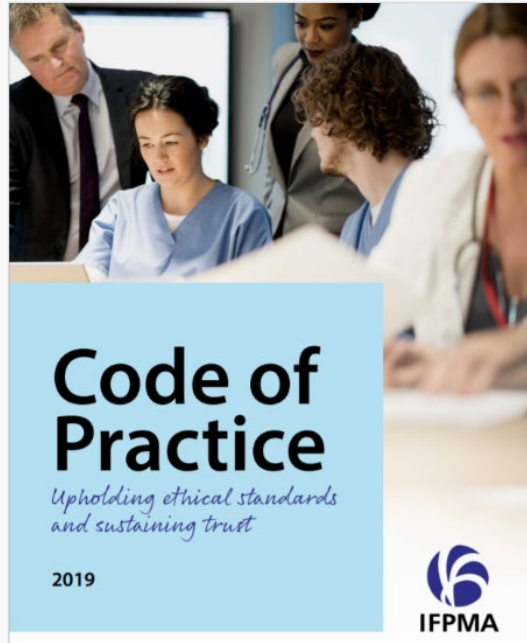
Despite the increasing number and scope of patient involvement initiatives, there is **no accepted master framework** for systematic patient involvement”

# First steps to involving patients in the publications lifecycle



# Overcoming compliance concerns

*Discuss the Code (early) – communication is not promotion*



18 | IFPMA Code of Practice

### 3. Pre-Approval Communications and Off-Label Use

No pharmaceutical product shall be promoted for use in a specific country until the requisite approval for marketing for such use has been given in that country.

This provision is not intended to prevent the right of the scientific community and the public to be fully informed concerning scientific and medical progress. It is not intended to restrict a full and proper exchange of scientific information concerning a pharmaceutical product, including appropriate dissemination of investigational findings in scientific or lay communications media and at scientific conferences. Nor should it restrict public disclosure of information to stockholders and others concerning any pharmaceutical product, as may be required or desirable under law, rule or regulation.

“

*FDA: “It has long been FDA policy not to consider a firm’s presentation of truthful and non-misleading scientific information about unapproved uses at medical or scientific conferences to be evidence of intended use when the presentation is made in non-promotional settings and not accompanied by promotional materials...”*

FDA Memorandum – Public Health Interests and First Amendment Considerations...January 2017; p 21.

”

# FDA perspective on involving patients in our publications



## 15th Annual Meeting of ISMPP

Communicating Science in an Era of Innovation and Change

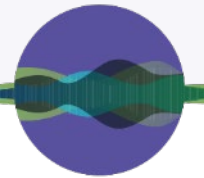
April 15-17, 2019 • Gaylord National Resort & Convention Center • National Harbor, MD, USA



FDA speaker **encouraged** industry and publication professionals to continue to involve patients in drafting and publishing articles

Chinyelum (Chi-Chi) Olele, PharmD, CDR, USPHS  
Patient Engagement Advisory Committee (PEAC) Manager  
FDA's Center for Devices and Radiological Health (CDRH).

# First steps to involving patients in the publications lifecycle





# Patients are already authoring peer-reviewed publications. They are meeting authorship criteria set by:

General Medical Journals

Specialist Journals

Industry-focused Journals

Patient-focused Journals

**RESEARCH**

**Shared decision making in patients with low risk chest pain: prospective randomized pragmatic trial**

Esik P Hess,<sup>1,2,3,4</sup> Judd E Hollander,<sup>5</sup> Jason T Schaffer,<sup>6</sup> Jeffrey A Kline,<sup>7</sup> Carlos A Torres,<sup>8</sup> Deborah B Dieckes,<sup>9</sup> Russell Jones,<sup>10</sup> Kelly P Owen,<sup>11</sup> Zachary F Meisel,<sup>12</sup> Michel Demers,<sup>13</sup> Annie LeBlanc,<sup>12</sup> Niley D Shah,<sup>14</sup> Jonathan Inselman,<sup>15</sup> Jeph Herrin,<sup>16</sup> Ana Castaneda-Guarderas,<sup>17,18</sup> Victor M Montori<sup>19</sup>

**ABSTRACT**

**OBJECTIVE** To compare the effectiveness of shared decision making with usual care in choice of admission for observation and further cardiac testing or for referral for equivalent evaluation to patients with possible acute coronary syndrome.

**DESIGN** Multicenter pragmatic parallel randomized controlled trial.

**SETTING** Six emergency departments in the United States.

**PARTICIPANTS** 898 adults aged ≥18 years with a primary complaint of chest pain who were being considered for admission to an observation unit for cardiac testing (45 were allocated to the decision aid and 847 to usual care), and 301 emergency clinicians (emergency physicians, nurse practitioners, and physician assistants) caring for patients with chest pain.

**INTERVENTIONS** Patients were randomly assigned (1:1) by an electronic, web-based system to shared decision making facilitated by a decision aid or to usual care. The primary outcome, selected by patient and caregiver advised, was patient knowledge of their risk for acute coronary syndrome and options for care, secondary outcomes were involvement in the decision to be admitted, proportion of patients admitted for cardiac testing, and the 30-day rate of major adverse cardiac events.

**WHAT IS ALREADY KNOWN ON THIS TOPIC** Current clinical, electrocardiographic, and laboratory data do not identify all patients with acute coronary syndrome who present to the emergency department, resulting in a 1.3% miss rate.<sup>1</sup> Given the potential medical, legal, and psychological sequelae associated with missing such a diagnosis, clinicians have a low threshold to admit patients for prolonged observation and advanced cardiac testing.<sup>2</sup> As a consequence, low risk patients are often admitted for observation and cardiac stress testing or coronary computed tomographic angiography (CTCA). This results in unnecessary hospital admissions,<sup>3</sup> false positive test results, and unnecessary invasive diagnostic investigations, at an estimated cost to the health-care system of over \$75 US/bp, 6h, 30 annually.<sup>4</sup> To avoid this cost, and patients with possible acute coronary syndrome in making risk informed shared

**ORIGINAL ARTICLE - CANCER RESEARCH**

**Factors influencing adherence in CML and ways to improvement: Results of a patient-driven survey of 2546 patients in 63 countries**

Jan Geisler,<sup>1,2,3,4</sup> Gloria Scharf,<sup>5</sup> Felice Bombaci,<sup>6</sup> Mina Dahan,<sup>7</sup> Jan De Jong,<sup>8</sup> Tony Gavri,<sup>9</sup> Jana Pelouchova,<sup>10</sup> Enzebisur Dziravik,<sup>11</sup> Joerg Hasford<sup>12</sup>, Verena Sophia Hoffmann<sup>13</sup>

**Abstract** Optimal adherence to CML therapy is of key importance to maximize treatment effectiveness. Two clinical studies (ADMG0 and Hammer04) have proven a clear correlation between adherence and achieving optimal treatment response and have revealed that non-adherence is common in CML patients (Marin et al. in *J Clin Oncol* 28(24):2318-2328, 2010; Niens et al. in *Hematologica* 99(3):437-447, 2014). The aim of this study is to assess the extent of suboptimal adherence and to investigate motivations and behavioral patterns of adherence in a worldwide patient sample. Questionnaires were provided by the CML Advocates Network and were filled in by patients online and offline. Patient characteristics, treatment and motivations were collected. Adherence was assessed by the 8-item Morisky Medication Adherence scale. Logistic regression models were fitted to investigate the influence of different factors on adherence. Overall, 2 546 questionnaires from 63 countries and 79 CML patient organizations were evaluable. 32.7% of participants were highly adherent, 46.5% were in the medium and 20.7% in the low adherence group. Factors increasing the probability of being in the high adherence group are older age, male sex, management of side effects, only one tablet per day and feeling well informed about CML by the doctor. More than 2 years since diagnosis were significantly lowering the chance as was the use of reminding tools. Living arrangements, multiple medication and personal payment obligations increased the probability to be at least in the medium adherence group. This is the most comprehensive study conducted to date to gain knowledge about factors causing non-adherence in CML. Better information on the disease, medication and management of side effects, supported by haematologists, is key to improve adherence.

**Keywords** CML treatment adherence - Patient-driven survey - Adherence patient motivations - Optimal adherence - Morisky Medication Adherence scale - Factors causing nonadherence - Tyrosine kinase inhibitors - Molecular response - Driving factors of nonadherence - Haematology - Chronic myeloid leukaemia - Patient advocacy - Behavioural patterns of adherence

**Special Populations: Review**

**Partnering With Patients in the Development and Lifecycle of Medicines: A Call for Action**

Anton Hoos, MD<sup>1</sup>, James Anderson, MA, MBA<sup>2,3</sup>, Marc Boutin, JD<sup>3</sup>, Lode Dewulf, MD, Dip Pharm Med, FFPM<sup>4</sup>, Jan Geisler, M, Marilyn Metcalf, PhD<sup>5</sup>, Graeme Johnston, LLB, IPFA<sup>6</sup>, Angelika Joos, MPharm<sup>7</sup>, Marilyn Metcalf, PhD<sup>8</sup>, Jeanne Regnante, MS<sup>9</sup>, Ifeanyi Sargeant, DPhil<sup>10</sup>, Roslyn F. Schneider, MD, MSc<sup>11</sup>, Veronika Todaro, MPH<sup>12</sup>, and Gervais Tougas, MD, CM<sup>13</sup>

**Abstract** The purpose of medicines is to improve patients' lives. Stakeholders involved in the development and lifecycle management of medicines agree that more effective patient involvement is needed to ensure drug patient needs are identified and met. Despite the increasing number and scope of patient involvement initiatives, there is no accepted/practice framework for systematic patient involvement in industry-led medicines research and development, regulatory review, or market access decisions. Patient engagement is very productive in some indications, but inconsistent and fragmentary on a broader level. This often results in inefficient drug development, increasing evidence requirements, lack of patient-centered outcomes that address unmet medical needs and tolerate adherence, and consequently lack of required specific costs and high costs to society and involved patients. Improved patient involvement can drive the development of innovative medicines that deliver more relevant and impactful patient outcomes and make medicine development faster, more efficient, and more productive. It can lead to better prioritization of early research, improved resource allocation, improved trial protocol designs that better reflect patient needs, and by addressing potential barriers to patient participation, enhanced recruitment and retention. It may also improve trial conduct and lead to more focused, economically viable clinical trials. At launch and beyond, systematic patient involvement can also improve the ongoing benefit-risk assessment, ensure that public funds promote medicines of value to patients, and further the development of the medicine. Progress toward a universal framework for patient involvement requires a joint, precompetitive, and international approach by all stakeholders, working in true partnership to consolidate outputs from existing initiatives, identify gaps, and develop a comprehensive framework. It is essential that all stakeholders participate to drive adoption and implementation of the framework and to ensure that patients and their needs are embedded at the heart of medicines development and lifecycle management.

**Keywords** patient involvement, medicines development

**Introduction: Problem Statement** Drug development times are around 10 to 15 years<sup>1,2</sup> and costs to bring a single new therapy to market are substantial.<sup>3,4</sup> From the industry perspective, not putting the correct medical needs of patients first, early in the development process, can lead to wrong priorities, wrong decisions on research design, and potentially costly late-stage failure. The complexity of clinical trials may lead to long and difficult experiences for patients<sup>5,6</sup> and recruitment into clinical trials is ever more competitive and increasingly problematic.<sup>7</sup> Many trials fail to achieve recruitment targets because they may be too restrictive in terms of exclusion/inclusion criteria, may impose an unfeasibly heavy

**EDITORIAL**

**Research Involvement and Engagement: reflections so far and future directions**

Richard Stephens<sup>1</sup> and Sophia Staniszewska<sup>2</sup>

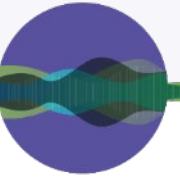
**Plain English summary** Two years ago we launched *Research Involvement and Engagement (RIE)* as an interdisciplinary co-produced journal, focusing on patient and wider involvement and engagement in all stages of health and social care research. In this Editorial we reflect on progress and consider future directions. Now indexed in PubMed Central, RIE's prime objective is to publish papers that report public involvement in enough depth to generate a sound and robust evidence base, from which others can draw to develop best practice. Our open access publishing enables anyone who wants to read a paper to access it free of charge, a powerful way of making research more open and more democratic, with RIE a key part of this slow but necessary evolution. While we have made progress, there is still a long way to go to embed involvement and engagement as normal within research practice. Publishers and funders have a vital role to play in changing research so the co-production of knowledge becomes the norm. In this Editorial we highlight key areas that we need to develop to strengthen involvement and engagement. We draw strength from knowing we are not alone in this journey. Our Editorial Board, our authors, our reviewers, and you dear readers, are all companions on this journey, making a wide range of contributions that help us move forward, slowly but surely.

**Abstract** Two years ago we launched *Research Involvement and Engagement (RIE)* as an interdisciplinary co-produced journal, focusing on patient and wider involvement and engagement in all stages of health and social care research. In this Editorial we reflect on progress and consider future directions. Now indexed in PubMed Central, RIE's prime objective is to publish papers that report public involvement in enough depth to generate a sound and robust evidence base, from which others can draw to develop best practice. Our open access publishing enables anyone who wants to read a paper to access it free of charge, a powerful way of making research more open and more democratic, with RIE a key part of this slow but necessary evolution. While we have made progress, there is still a long way to go to embed involvement and engagement as normal within research practice. Publishers and funders have a vital role to play in changing research so the co-production of knowledge becomes the norm. In this Editorial we highlight key areas that we need to develop to strengthen involvement and engagement. We draw strength from knowing we are not alone in this journey. Our Editorial Board, our authors, our reviewers, and you dear readers, are all companions on this journey, making a wide range of contributions that help us move forward, slowly but surely.

Industry co-authors included Pfizer, Novartis, UCB Pharma, GSK, Merck (passed 'compliance concern' barrier)

# Evidence

## World-first systematic review... with patient authors



15<sup>th</sup> ANNUAL MEETING OF ISMPP

### SYSTEMATIC REVIEW AND EVIDENCE-BASED RECOMMENDATIONS FOR INVOLVING PATIENTS AS PUBLICATION AUTHORS

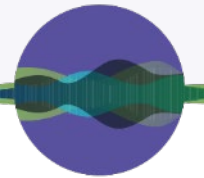
**Plain language title: How to involve patients as authors**

Professor Karen L. Woolley PhD ISMPP CMPP™  
Global Lead, Patient Partnerships  
Envision Pharma Group

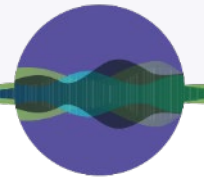
 Dr Lauri Arnstein Envision Pharma Group	 Richard Stephens Consumer Forum, National Cancer Research Institute	 Kawaldip Sehmi International Alliance of Patients' Organizations
 Arabella Sargent Envision Pharma Group	 Anne-Clare Wadsworth Envision Pharma Group	 Prof. Beverley Yamamoto Hereditary Angioedema Japan
 Rachel Jones Swiich Health	 Prof. Karen Woolley Envision Pharma Group	 <i>Acknowledge:</i> Dr Tom Gegeny Envision Pharma Group

Selected by industry peers (via blinded peer review) for an oral presentation at the 15<sup>th</sup> Annual Meeting of ISMPP  
21 Evidence-based recommendation, before during and after manuscript preparation

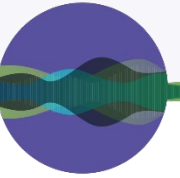
# First steps to involving patients in the publications lifecycle



# First steps to involving patients in the publications lifecycle



# Plain language summaries can empower patients in their discussions with HCPs



- Access to PLS can give patients a sense of empowerment<sup>1</sup>
- In a survey of patients and caregivers, 81% felt that PLS would help them to discuss treatment options with HCPs<sup>2</sup>

## VALUE TO PATIENTS<sup>3</sup>

*“The more informed a patient is, the better the conversation they can have with their doctors.”*

## VALUE TO DOCTORS<sup>3</sup>

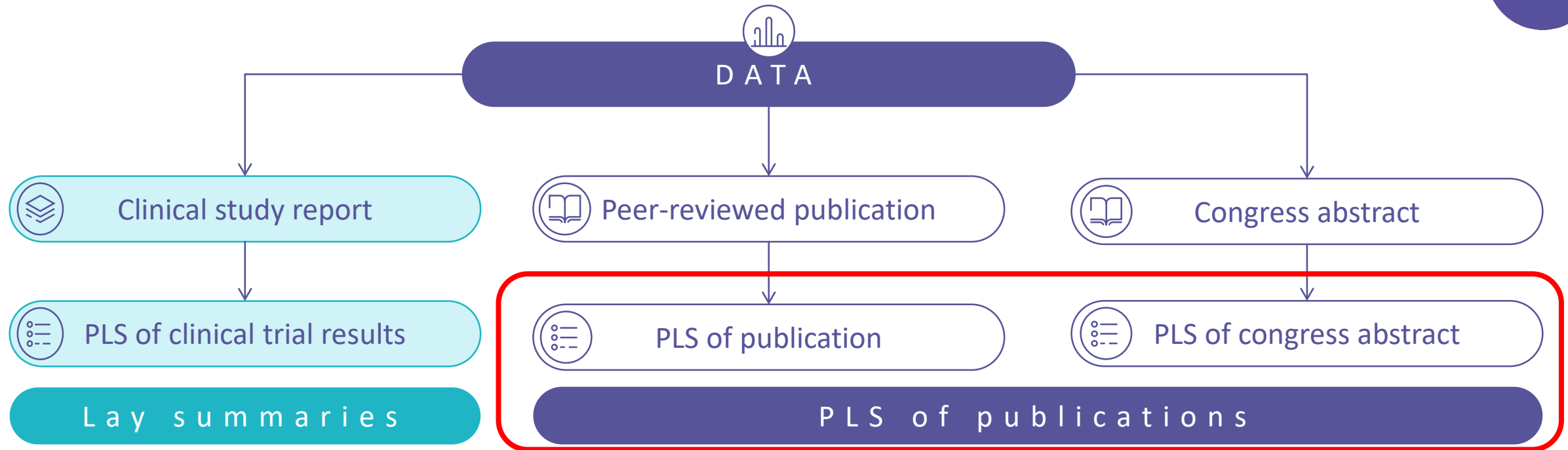
*“[Use of a plain-language summary] could help generate dialogue, increase efficiency and streamline communication between HCPs and patients.”*

1. McKinnon VE. Poster presentation at EMBLIVE 2019.

2. Georgieva A et al. Poster presentation at ISMPP EU Meeting 2018.

3. Pushparajah DS et al. *Ther Innov Regul Sci*. 2017; <https://doi.org/10.1177/2168479017738723>.

# Three types of summaries broaden data dissemination: two relate to publications



- Mandated by the EU Clinical Trials Regulation No. 536/2014 – will be housed in the EU database (2020 onwards)<sup>1,2</sup>
- The FDA recognises their importance but does not mandate them<sup>3</sup>

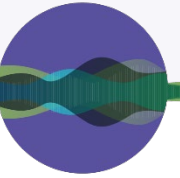
- Not currently mandated
- Increasingly being explored by journals and industry
- Cover a much broader range of evidence types than clinical trial results lay summaries

1. European Commission. Clinical trials - Regulation EU No 536/2014. [https://ec.europa.eu/health/human-use/clinical-trials/regulation\\_en](https://ec.europa.eu/health/human-use/clinical-trials/regulation_en). [Accessed February 7, 2019].

2. European Medicines Agency. Clinical trial regulation. [http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general\\_content\\_000629.jsp](http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000629.jsp). [Accessed February 7, 2019].

3. Food and Drug Administration. Draft FDA guidance on provision of plain language summaries. <https://mrctcenter.org/wp-content/uploads/2017/06/2017-06-13-MRCT-Draft-FDA-Guidance-Return-of-Aggregate-Results.pdf> [Accessed October 8, 2019]

# Increasing numbers of journals are including PLS



## *FREQUENCY OF PLS PUBLISHING IS LOW, BUT INCREASING<sup>1</sup>*

- In a review of ~7630 journals, **<1% published PLS** alongside the main abstract<sup>1</sup>
- Many journals currently publishing are **newly established** and have always included PLS in their formats<sup>1</sup>
- Submitting a PLS as **supplementary information** can provide more layout options that may improve readability



■ BMC

Research Involvement and Engagement



**BJOG**

An International Journal of  
Obstetrics and Gynaecology



**PLOS** | MEDICINE



**Cochrane  
Library**

1. Houghton M, Machin D. Poster presentation at ISMPP EU Meeting 2017.

# Journal variation in *what, who, when, where*

Assessment of 10 journals from different publishers identified as having PLS using eLIFE

## Terminology

**9** different terms for PLS were found

*Annals of the Rheumatic Diseases* refers to both 'patient summaries' and 'lay summaries'; *Autism Research* has changed from 'lay abstracts' to 'scientific summaries for families with ASD', and more recently to 'lay summaries'

Some terms do not intuitively make the intended audience clear (eg 'significance statement', 'author summary'), meaning lay readers may overlook them

## Requirements

Are PLS developed by authors?



- No - by editors (based on author responses to questions)
- Yes - although sometimes by editorial team
- Yes - always

When are PLS required?



- At acceptance
- At revision
- At submission

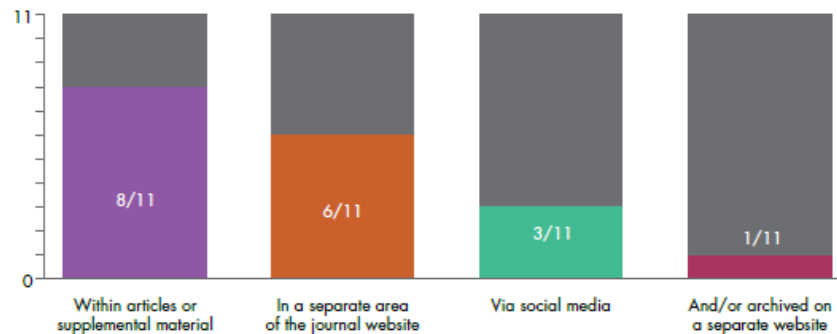
Are PLS required for all research articles?



- No - only where PLS are volunteered by authors
- No - only those selected by editors
- Yes - all articles/all research articles

## Location

The sharing mechanism/location of PLS varies:



## Accessibility

All PLS are **freely accessible**, with the exception of *ACS Infectious Diseases* - e-mail follow-up determined that these PLS are only for the press, and are not publicly available  
 PLS published within articles are freely accessible, even when the main article sits behind a paywall

## PubMed visibility

Are PLS noted on PubMed?



- PLS provided on PubMed, alongside conventional abstracts
- No indication of PLS availability



# The PLS of Publications Toolkit

Launch of the world's first PLS of Publications Toolkit  
Supported by Envision's partner, Patient Focused Medicines Development,  
and co-created with patients, publishers, editors, industry

Winner, 'Best Practice' Award,  
at ISMPP Annual Meeting 2019 and  
selected for Guided Poster Tour

ENVISION PHARMA GROUP PATIENT FOCUSED MEDICINES DEVELOPMENT

Home PLS Toolkit Contact Us

## Plain Language Summaries (PLS) of Publications Toolkit

A best-practice resource for PLS of peer-reviewed publications and congress abstracts

Explore the Toolkit >

#ISMPP15AM

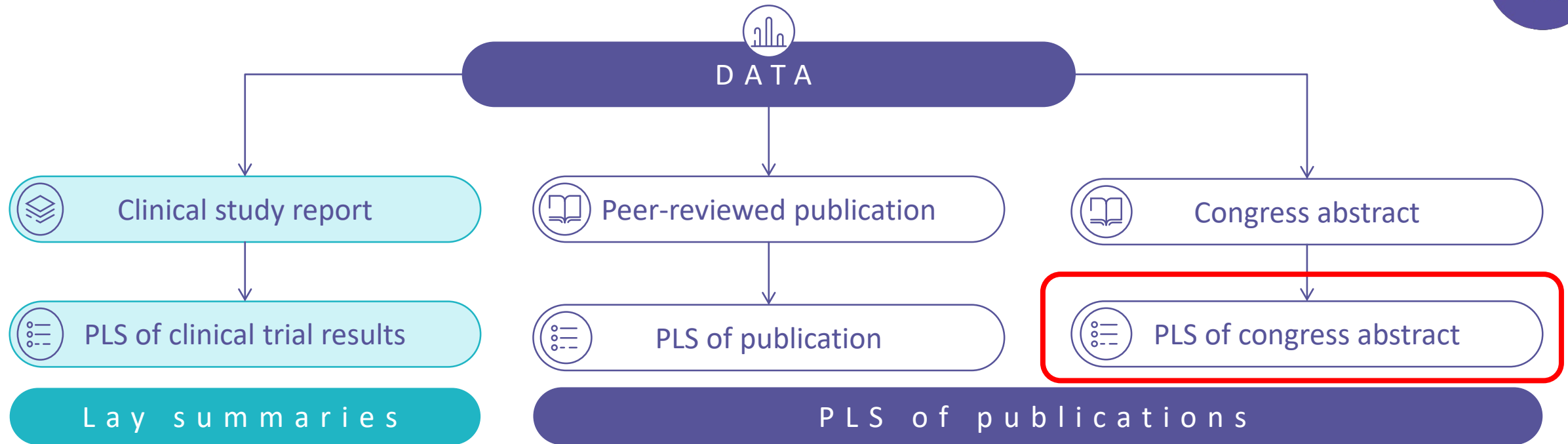
### BEST PRACTICE AWARD FOR THE POSTER PRESENTATION

Plain language summaries of publications:  
Addressing the HOW via stakeholder survey and workshop

ENVISION PHARMA GROUP  
Driven by evidence, enabled by technology

<https://www.envisionthepatient.com/plstoolkit/>

# Three types of summaries broaden data dissemination: two relate to publications



- Mandated by the EU Clinical Trials Regulation No. 536/2014 – will be housed in the EU database (2020 onwards)<sup>1,2</sup>
- The FDA recognises their importance but does not mandate them<sup>3</sup>

- Not currently mandated
- Increasingly being explored by journals and industry
- Cover a much broader range of evidence types than clinical trial results lay summaries

1. European Commission. Clinical trials - Regulation EU No 536/2014. [https://ec.europa.eu/health/human-use/clinical-trials/regulation\\_en](https://ec.europa.eu/health/human-use/clinical-trials/regulation_en). [Accessed February 7, 2019].

2. European Medicines Agency. Clinical trial regulation. [http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general\\_content\\_000629.jsp](http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000629.jsp). [Accessed February 7, 2019].

3. Food and Drug Administration. Draft FDA guidance on provision of plain language summaries. <https://mrctcenter.org/wp-content/uploads/2017/06/2017-06-13-MRCT-Draft-FDA-Guidance-Return-of-Aggregate-Results.pdf> [Accessed October 8, 2019]

# PLS of congress abstracts (APLS)

## THE CHALLENGE

- Patients are demanding access to the latest scientific information
  - Becoming more involved at congresses (eg, ASCO, ESMO)
- Pharma client wanted to address this unmet need
  - demonstrate a compliant, tangible commitment to patient involvement

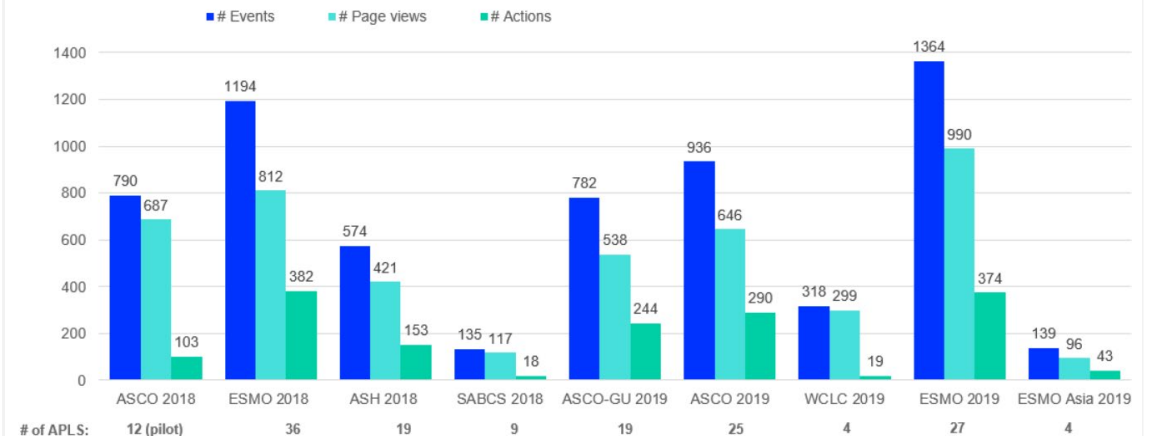
## THE SOLUTION

- Accessible, understandable, usable APLS
- Scan QR code to access PLS
- View PLS on a device
  - Menu options to:
    - Download PLS
    - Print PLS, or
    - Access original scientific abstract (redirected to the congress website)
- An information sheet listing the titles and hyperlinks to the full APLS are made available to patient advocates if permitted by congress
- Press release and news article on company website

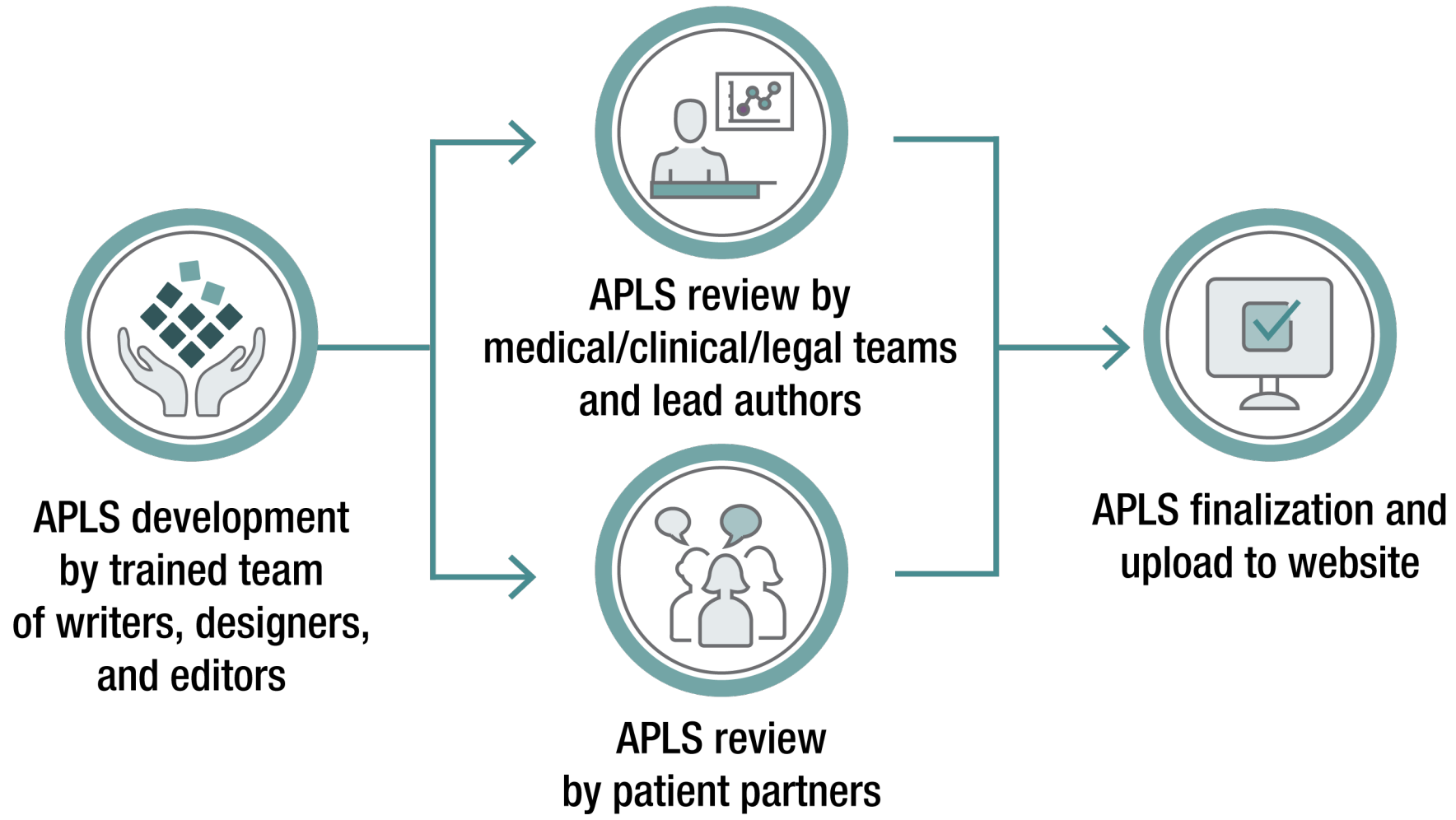
## IMPACT: Every APLS was accessed

185 APLS have been co-developed with patients and authors

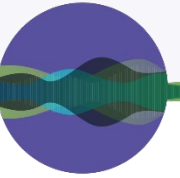
- Over 4600 page views
- Over 1600 additional actions



# PLS of congress abstracts (APLS)



# APLS practical considerations



## Source

- Abstract vs poster
- Which abstracts?



## Appropriate language, discordant comments

- Plain language vs scientific
- Widely understandable vs accuracy
- Consistency: across abstracts, across meetings
- Glossary



## Training

- Agency, different type of writing for MWs
- Pharma company
- Authors
- Patients



## Final adjudication



## Timeline

- Draft text
- Graphics
- Data QC
- Reviews
- Layout



# Conclusions



Patients are authoring peer-reviewed publications.

- Evidence-based recommendations can help us to minimise risks and maximise benefits

Key considerations include

- Keeping to plain language
- Consistency across outputs
- Timelines

Interest from publishers, pharma and patients is increasing

Practical tools and guidance are available to support you

Plain language summaries are an 'easy' first step to involving patients in publications