

Building trust in RWE

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Introduction

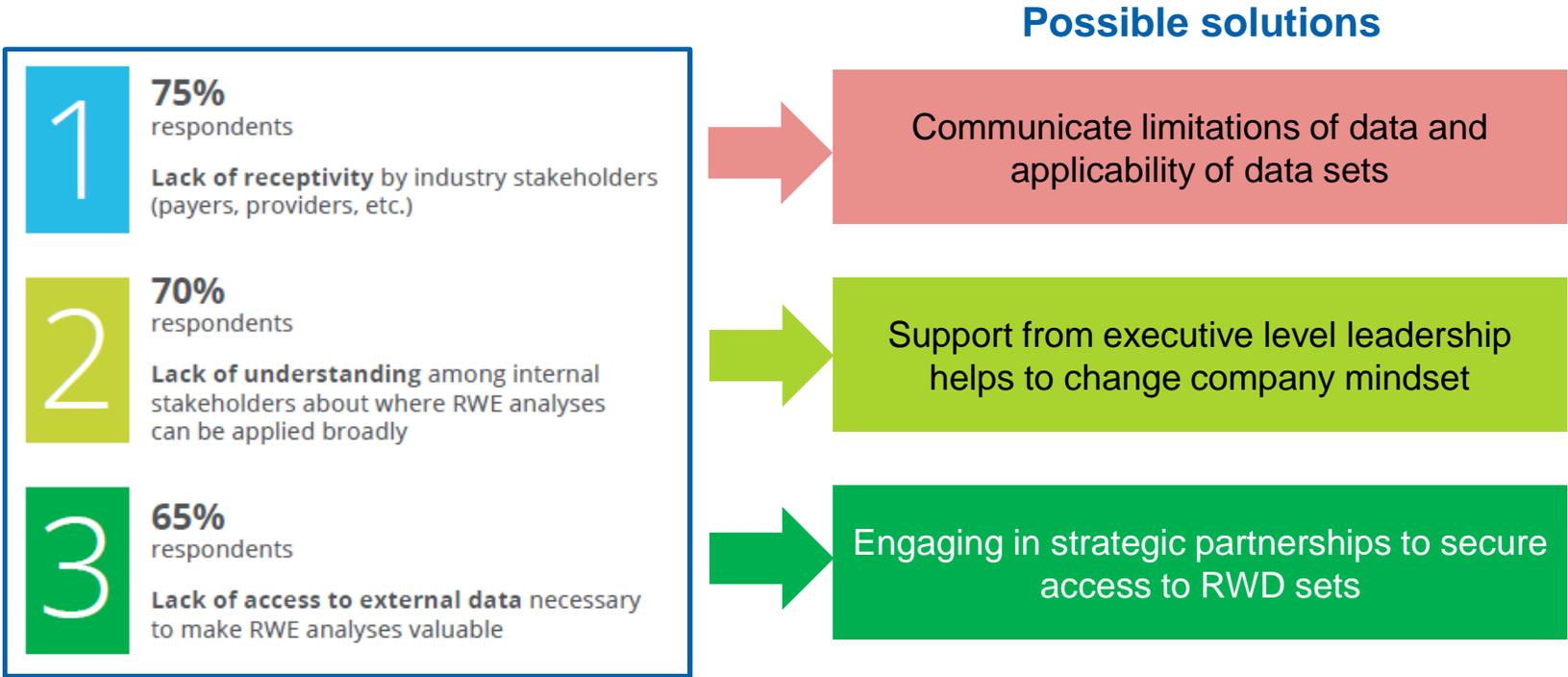
Aim

To explore the perceived value, capabilities and barriers to using RWE

- The Deloitte RWE Benchmarking Survey provides **insights into the life science industry's RWE capabilities**
- The survey reveals emerging trends in **RWD sources** used, **when RWE is used**, areas of **biopharma investment**, and internal and external **barriers to RWE success**

Main challenges for RWE

What are the top three challenges to the success of your RWE efforts?



The chart shows the top three barriers calculated as the percentage of respondents who ranked the barrier in the top three.

[Deloitte's 2018 RWE Benchmarking Survey](#)

Introduction



RWE is increasingly required by regulators, payers and physicians to support decisions over the licensing, reimbursement and use of our medicines



Open, truthful, balanced and non-misleading dialogue about how our medicines work in real clinical practice is crucial



However, there are challenges to overcome; stakeholders often have concerns with, or do not trust, RWE (and are less familiar with RWE than with RCTs)

Considerations for communicating RWE: communication strategy



Design and implement an **effective communication strategy** that ensures predetermined messages reach the target audiences, including regulators, payers, providers, patients and HTA agencies



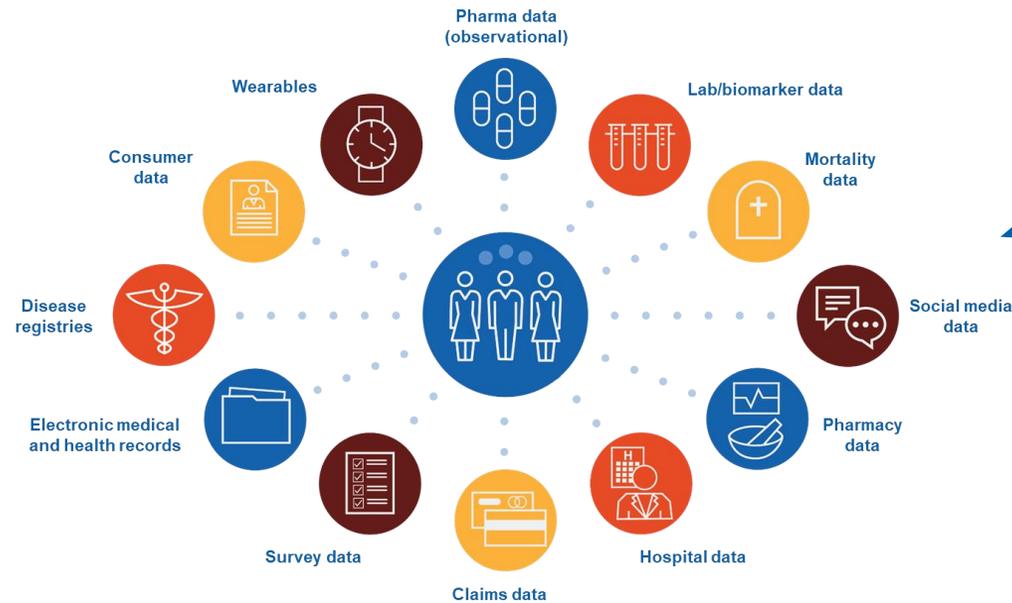
Appreciate the drivers of different stakeholders, which will determine how evidence can be communicated



Be aware that an **effective communication strategy** is just as important as designing and executing the study

What is RWE?

- Evidence used for **clinical, access and payment decision-making** that is generated from data **not collected in conventional controlled clinical trials**^{1,2}



“90% of the world's data have been produced in just the last two years”

(US Chamber of Commerce Foundation)

1. [Garrison et al. Value Health 2007;10:326–35](#)
2. Novartis internal discussions

Big data, RWD and RWE

RWD

RWD are **any data that are not captured within the context of a conventional clinical trial** and are **not explicitly intended for research purposes**

- Supplements to support RCTs, registries, practical clinical trials, claims databases/administrative data, health surveys, EMRs, medical chart reviews
- Outcomes (e.g. clinical, HRQoL, PROs, economic)

Big data

RWD are considered 'big data' when **vast in quantity and multiple sources are combined**

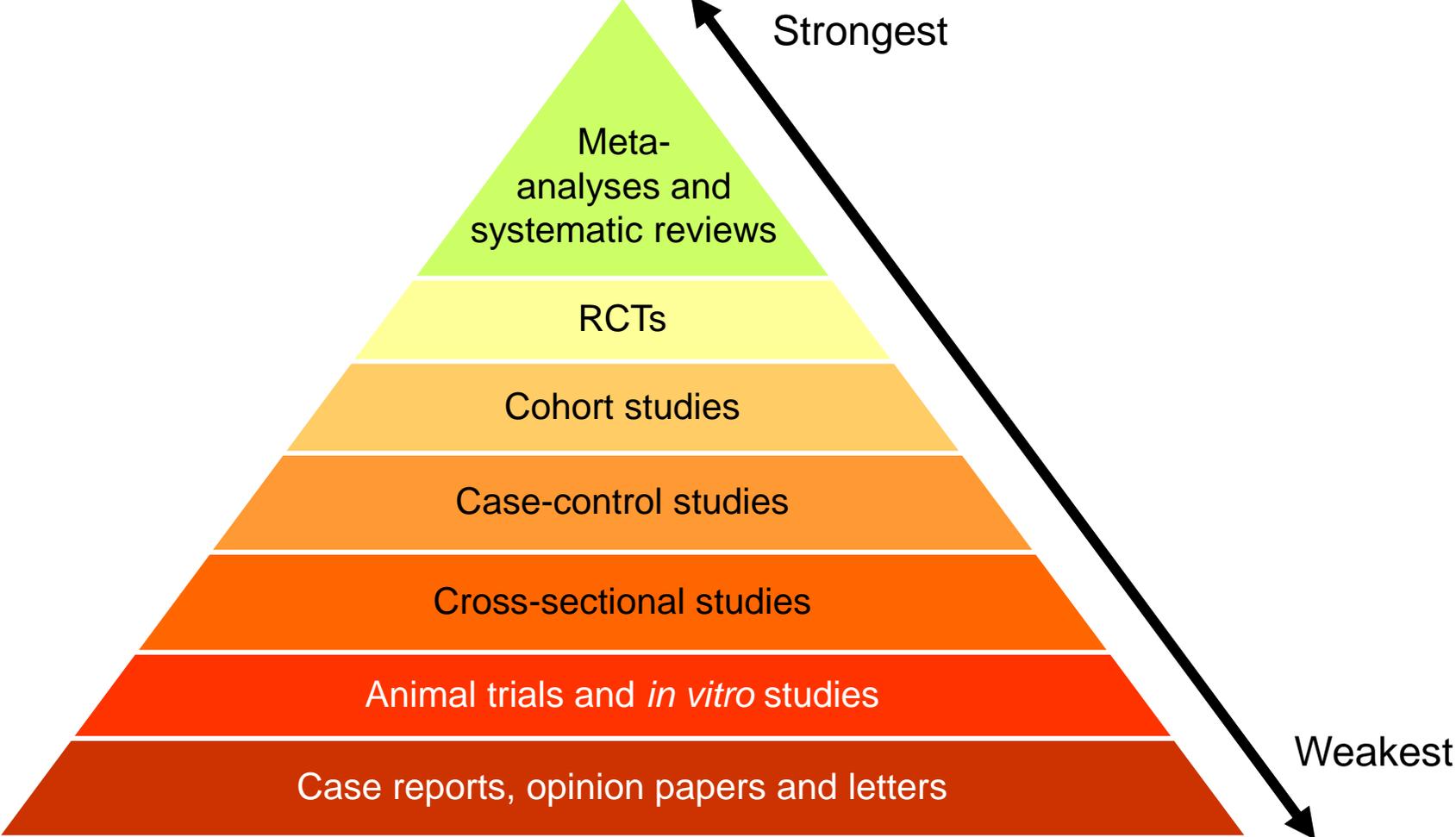
- RWD are often considered to be a form of big data, not because of the volume (they are relatively small in size when compared with big data), but because of their complexity and diversity

Differences between RCTs and RWE studies

	RCTs	RWE studies
 Outcome assessed	Efficacy, safety	Real world effectiveness and safety, HRQoL, costs, resource use, PROs, patient preference, adherence etc.
 Tightly controlled?	✓	✗
 Higher internal validity?	✓	✗
 Higher external validity?	✗	✓
 Longer follow-up?	✗	✓
 Greater patient numbers?	✗	✓
 Data collection	Primary, prospective	Primary or secondary, prospective or retrospective
 Familiarity to decision makers	High	Lower

HRQoL, health-related quality of life; PRO, patient-reported outcome; RCT, randomized controlled trial.

Evidence hierarchy is not always relevant

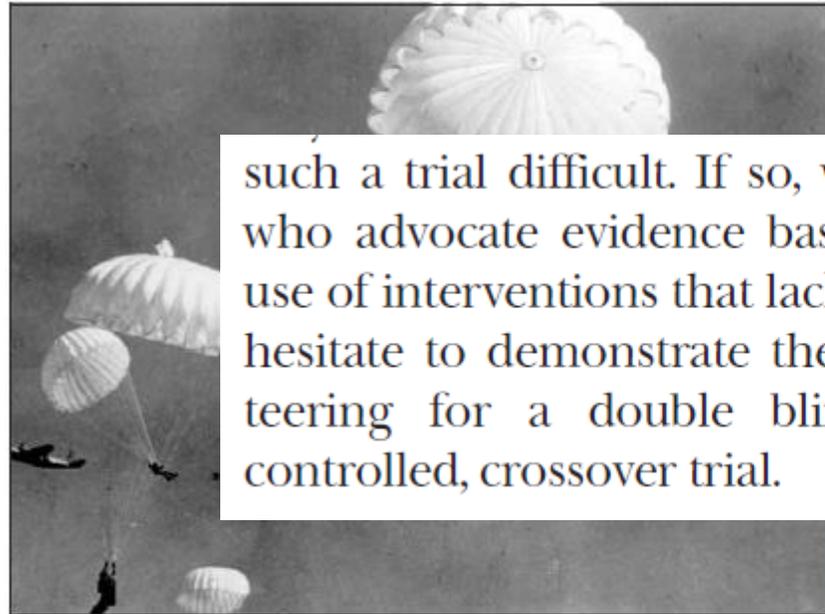


RCT, randomized controlled trial.

Why evidence-based medicine isn't everything

Parachute use to prevent death and major trauma related to gravitational challenge: systematic review of randomised controlled trials

Gordon C S Smith, Jill P Pell



such a trial difficult. If so, we feel assured that those who advocate evidence based medicine and criticise use of interventions that lack an evidence base will not hesitate to demonstrate their commitment by volunteering for a double blind, randomised, placebo controlled, crossover trial.

Parachutes reduce the risk of injury after gravitational challenge, but their effectiveness has not been proved with randomised controlled trials

Abstract

Objectives To determine whether parachutes are effective in preventing major trauma related to gravitational challenge.

Design Systematic review of randomised controlled trials.

Data sources: Medline, Web of Science, Embase, and the Cochrane Library databases; appropriate internet sites and citation lists.

Study selection: Studies showing the effects of using a parachute during free fall.

major trauma,
> 15.

any randomised
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of parachutes has

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benefit if the most

data. We think that everyone might benefit if the most radical protagonists of evidence based medicine organised and participated in a double blind, randomised, placebo controlled, crossover trial of the parachute.

Stakeholder concerns surrounding RWE

Concerns with/distrust of RWE may occur as a result of methodological challenges

1

Lack of randomization

- Observational nature means groups cannot be matched for characteristics, which could lead to bias

2

Representativeness of results

- Results may not represent the wider population owing to selection of unsuitable data sources, inappropriate algorithms for identification, etc.

3

Publication Bias

- How can study teams dispel the suspicion that multiple similar studies were run, but only the one that gave a positive result was published?

4

Conflicting or contradictory results

- Different studies can show different outcomes as a result of the plethora of data sources and analytical approaches that can be used

Building trust in RWE through communication and transparency



Involve stakeholders early
e.g. obtain buy-in/endorsement
at concept stage



Follow best practice guidance (e.g. NPC, AHRQ, PCORI, ENCePP, ISPE/ISPOR, STROBE)



Demonstrate transparency
in methodology



Communicate what has
been done to **address**
stakeholders' concerns



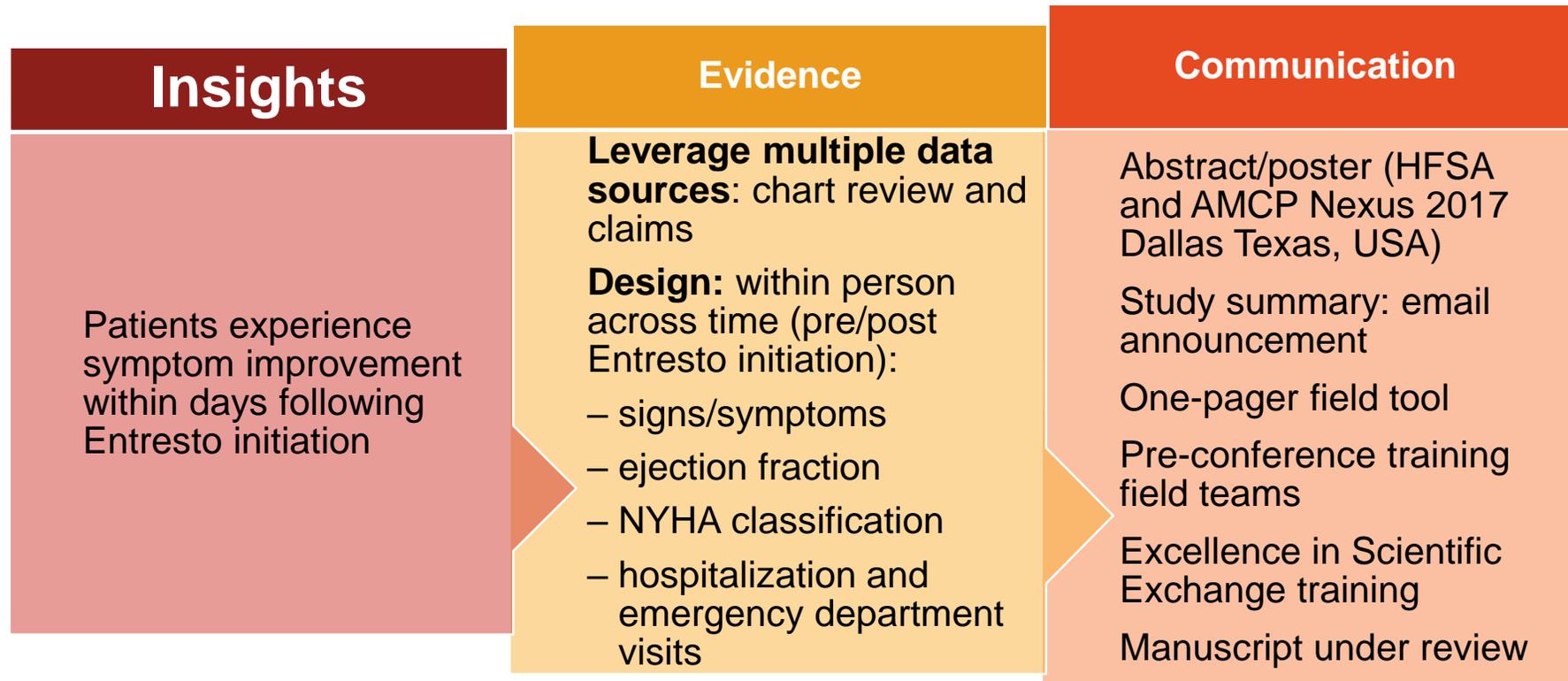
Commit to publishing
results of all studies

Insight vs feedback

- **Insight**
 - The capacity to gain an accurate and deep understanding of someone or something
- **Feedback**
 - Information about reactions to a product, or a person's performance of a task

Case study: Humana study

Signs and symptoms of heart failure



Case study: burden of PsO in Portugal (PeSsOa)



- Low disease awareness
- No available data on disease burden and QoL
- Difficult to access biologics in Portugal
- Long waiting time to visit a dermatology specialist in the public sector
- Close interaction with PAG (PSOPortugal)
- Need for integration of patients' voices in disease awareness and advocacy initiatives with different stakeholders



Case study: burden of PsO in Portugal (PeSsOa)



- ✓ Describe the Portuguese population with PsO
- ✓ Generate data on the burden of PsO, capturing the patient perspective
- ✓ Evaluate the impact of PsO on patients' QoL and social/day-to-day activities
- ✓ Quantify direct and indirect economic burden
- ✓ Characterize patient access to healthcare and use of health resources



Case study: burden of PsO in Portugal (PeSsOa)



Solution

- Observational, cross-sectional study
- Adult patients with PsO
- Electronic questionnaire completed directly by participating patients
- Dissemination of the questionnaire
 - PSOPortugal website and Facebook
 - Study physicians/HCPs
 - NVS website
- Recruitment period: June 1–July 31 2016

Case study: burden of PsO in Portugal (PeSsOa)



Survey questions were developed by the Portuguese CPO (Patient Relations, Medical, HEOR) in collaboration with the PAG



The survey was revised by the scientific committee (three dermatologists)



Worked with local university pharmacy faculty who supported the protocol and performed the analyses

Case study: burden of PsO in Portugal (PeSsOa)



Results



631 surveys completed;
564 surveys analyzed



72% were active
workers



52% were
followed up in a
private setting



Mean \pm SD age was 44
 \pm 13.7 years; 68% were
aged < 50 years



Plaque PsO was the
most common type (79%)



Mean cost of clinical
follow-up appointment: €31.50



62% were female



Depression (25%), PsA
(22%) and hypertension
(18%) were the most
common comorbidities



Mean cost of medical
treatment: €20–€50/month



Most patients had a
high school degree



> 63% of patients had
moderate to severe PsO



25% reported severe/
extremely severe impact
on QoL

Case study: burden of PsO in Portugal (PeSsOa)



- Shared with patients at a national meeting (PSOPortugal)
- Shared with the Portuguese parliament's health committee on World Psoriasis Day 2017
- Leveraged results in the media
- Presented to the medical community at two national congresses (dermatology and rheumatology)
- Three publications at ISPOR 2016 and 2017
- Communication at a national health economic congress
- Three publications in development



Case study: burden of PsO in Portugal (PeSsOa)



Collaboration with stakeholders
(PAG, HCPs, academia)



Cross-functional collaboration
(Patient Relations, HEOR, Medical)



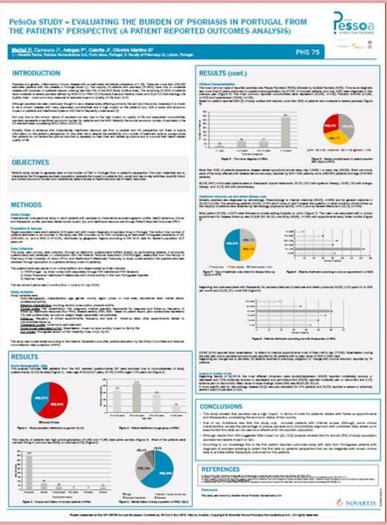
Broad communication plan (social media
drive, scientific events, congress,
publications, Portuguese parliament)

Case study: burden of PsO in Portugal (PeSsOa)



The collage features several pieces of media coverage from Portugal:

- Newspaper Article 1:** "PSORÍASE AFETA 200 MIL EM PORTUGAL" (Psoriasis affects 200,000 in Portugal). It discusses the prevalence of the disease and the impact on patients' quality of life.
- Newspaper Article 2:** "A VIDA ENCURTA CINCO ANOS" (Life is shortened by five years). A quote from Paulo Ferreira, a dermatologist, stating that psoriasis significantly impacts life expectancy.
- TV News Segment:** A news anchor reports on the national study "PeSsOa" (Estudo nacional para a Pessoa com Psoríase). The segment includes a quote from a patient and a doctor.
- Other Articles:** Various smaller articles related to health and chronic diseases, such as "SAÚDE COM O CM" and "DOENÇA CRÓNICA".



Thank you
