Health Care Research & Implementation

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Dear ladies and gentlemen, dear colleagues, dear readers,

What is evidence? Evidence conveys to us an objective fact, one bit of reality – it opens for us a window through which we can see a glimpse of the truth concerning a specific question. So evidence is a measure against inaccuracy, misconception, error, bias – at its best it defeats subjectivity, alternative facts, manipulation, vested interests, and lies. Evidence helps us to not readily accept authoritative statements, eminent colleagues' opinions, common beliefs, and, generally, things at face value.

That makes evidence the legitimate base of scientific reasoning, the founding principle of rational decision making based on science. It is valuable, and it is unique, in that it cannot be substituted by anything else.

Evidence cannot be generated. It is already there and only waits to be found – by careful observation, exact documentation, meticulous evaluation, comprehensive compilation and integration, repitition, and critical appraisal.

As health care researchers we want medical care to be evidence-based. And we are not alone. A valid basis for medical decision-making in Germany is a nationwide legal standard. In SGB V it states, that „The quality and efficacy of all services have to comply with the recognized standard of medical knowledge and needs to consider medical progress" (§ 2 para (1)).

While it is not the questions whether or not medical decisions require pertinent evidence, there is an ongoing debate on the ways and means to find this evidence. Health care researchers agree that evidence is nature's answer to a questions that we ask in experiments, in epidemiologic or in clinical studies. The operationalization however, that the only accepable design for proving efficacy in these studies is the randomized, controlled, double-blind clinical trial has reached its limits.

Medical progress is exponential and has led to an accelerating development of not only new drugs, but also of new diagnostic and therapeutic principles. Gene therapy, anti-sense RNA, monoclonal antibodies, and super specific small molecules directly adress pathogenic gene mutations, or interact with their specific gene products. Other lines of progress include biosimilars that closely resemble their physiological analogues and closely mimic their functions. In CAR-T therapy a patient's own immune cells are genetically manipulated to enable them to more effectively attack cancer clones.

When therapies become increasingly individualized, questions multiply exponentially, and needs for evidence galore. If decisions get complex, evidence needs to become more and more specific, and when problems are urgent, as in a pandemic crisis, we need this evidence fast. All these megatrends conflict with the traditional requirement of a completed positive RCT for each medical novelty to become effective in patient care.

In this issue of the Journal, Pfaff and Schmitt address this increasingly inescapable dilemma (Pfaff H, Schmitt J 2024 [1]). While they leave no doubt on the necessity of uncompromised evidence, they widen the horizon and discuss data, and evidence beyond RCTs. They quote David Sackett, who urged us to always base decisions on the best available evidence (including the patients' preferences

and the physicians' competencies) (Sackett D 1997 [2]) and stress other sources including high quality registries, obervational studies, and prospective data collection accompanying inroduction of a new therapy to monitor its effectiveness as well as associated risks while it is used on conditional terms in practical care.

Extending the scope of acceptable evidence does not compromise in itself any of the criteria for rational decision making. It rather extends the area where decisions can be closely informed by evidence – and supports that this should always be the most robust that we can use for a given decision. The collection and analysis of both primary data in hypothesis-based studies and secondary data reflecting the real world of care practice need special attention, skills, and experience. This puts health care researchers in a crucial position. Whereas the interpretation of a well designed RCT does not require neither extended statistical nor substantive training- both is urgently needed in the use of results based on data from within the health are system. Health care researchers need to comprehensively address all systematic as well as random shortcomings of the data, the limitations of the study designs, and the multitude of biases in the results. Heads up, colleagues, that is what we do all the time in our studies, what we have been trained for, and what we humbly concede to all RCT-proponents. There is just no alternative to health care research methodology and conceptional thinking in an increasing area of rational medical decision making.

We wish you an interesting time reading, thank you for your interest in our Journal – and are looking forward to hearing from you!

Wolfgang Hoffmann, Editor in Chief, Chairman DNVF
Martin Härter, Chief Executive Officer DNVF

Conflict of interest

The authors declare no conflict of interest.

References

[1] Pfaff H, Schmitt J. Shifting from theoretical best evidence to practical best evidence: an approach to overcome structural conservatism of

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