

The Patient Data Paradigm

Precision medicine and personalised treatment options have become a reality in treating cancer. However, with multiple challenges still to be overcome, it is vital to keep approaching patient contribution research from the patient's perspective

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Translational research, multifaceted collaboration, and next-generation trial designs are helping to progress the reality of precision medicine in cancer treatment. Patient data – in particular, genomic data and pharmacogenomic data – have become the centre point of research. However, unless we remember to view patient contribution in medical research with the patient's interest at heart, we may risk failing in the attempt to implement precious patient data effectively.

Cancer Therapy

Cancer therapy has seen one of the most revolutionary transformations in decades. A deeper understanding of the disease at a molecular level has shifted the cancer treatment paradigm, and personalised cancer medicines are now promising to deliver the right drug to the right person at the right time, tackling tumour behaviour with precise mechanisms of action.

Highly personalised, rationally designed, and biologically derived therapies are driving the future of adequate cancer care, and immunotherapies are being introduced alongside surgery, radiotherapy, and chemotherapy as standard care options. However, due to the still novel approach of facing these enormous challenges with targeted therapies, further research is extremely necessary.

Cancer Research

Despite its complex biological manifestation, cancer is a genetic disease of somatic cells: somatic mutations that can be described as a sequence of a small number of discrete genetic events. A genomic and biomarker approach to treating this disease is therefore widely applied. For quite some time, the focus of research has been on identifying genetic alteration and consequent abnormal/excessive production of proteins in tumours as druggable targets. However, tumours (even of the same type) behave and mutate differently in different patients.

Intra-patient and inter-patient tumour heterogeneity is the root cause of acquired drug resistance and a likely



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explanation of the wide disparity in patient outcomes. Moreover, drug toxicity remains a reality that needs attention. Despite consensus that targeted therapies are generally considered to be less toxic than conventional cytotoxic chemotherapy, sudden fatal adverse events do occur, and chronic low-grade toxicities tend to have a significant impact on the patients' quality of life.

Clinical trials aim to predict which tumour types are most responsive and which patients may benefit the most. Biomarker-driven trials are moving research forward. The future of targeted therapies will probably include more multimodality therapies that combine the immunological

approach with novel therapeutic molecules able to pharmaceutically trace protein-protein interaction targets.

Challenges

There are still enormous challenges to face in the implementation of novel and personalised treatment and research options. Oncogenomic research does not come cheap; the field needs to invest in highly sophisticated technology, and highly skilled data scientists have to collaborate alongside scientists. Moreover, the gathering and storage of enormous amounts of increasingly complex data weigh heavily on electronic systems.



Without having a clear idea of what needs to be done with this data, what problems need to be solved, and who is going to pay for the collection of this data, we are going to drown in data noise or end up generating data without scientific value.

Today's oncology professionals are requested to possess a series of added skills to carry out their work, and a lot of resources are invested in continuous and time-consuming training. In short, costs for research – in clinical trials, especially – continue to escalate. More specialised resources are needed to manage modern oncology trials, and selecting patients to participate in trials have become highly specialised. Therefore, patient data has become the centre point of next-generation clinical trials.

The reshaping of clinical trial design and the need to rethink clinical development strategies are inevitable consequences of implementing patient-centric drug development. Involving patients from the beginning, acknowledging their key importance in the process, and treating precious patient data with close attention have certainly become very important aspects to consider.

The aim to optimise patient contribution in medical research by putting patient interests at heart is steering the quest towards establishing better trial retention, better follow-ups, and better care options for cancer. With precision and personalised medicine, 'wasting' patients on control groups in trials will have to be replaced by smarter options.

The more we learn about cancer at a molecular level, the more we will need to redefine its classification. Patient subgroups are turning into more subgroups, and finding the right patients to benefit from a targeted therapy is only going to happen if we create possibilities that stratify large patient populations through integrated clinical trial structures.

A significant shift is happening with the implementation of next-generation clinical trials. Trials with an adaptive design often prove to be more efficient than classically designed trials because these tend to make better use of resources such as time and money, and they often require fewer trial subjects. Basket trials, umbrella trials, and combinations of both are examples of optimising resources and information gathering. Pragmatic trials using real world evidence are also introduced to make the most of patient data.

The Fundamental Role of Technology

The implementation of sophisticated platforms for translational research, as well as genomic-annotated databases, has become a reality. Artificial intelligence

and machine learning are widely used to handle and analyse increasingly complex amounts of data. Blockchain technology is promising to provide a transparent, decentralised, and secure future solution for existing issues related to data privacy, data authenticity, and data traceability.

Oncology Leading the Change

With Cancer Core Europe as a sure example, oncology professionals have demonstrated a positive willingness to establish multi-country collaboration and create an environment where quality-controlled data is shared with a scientific approach. This multi-stakeholder collaboration aims to establish a translational cancer research continuum that covers basic, preclinical, early to late clinical, and outcomes research.

Compared to 10 years ago, pharma companies have become increasingly transparent and are more collaborative with a much wider sphere of stakeholders. Researchers share data through specific therapeutic platforms, and have access and opportunities to comment on publications. Academic institutions have formed closer partnerships with healthcare institutions and nonprofit organisations, and patient associations are very active in spreading information about the latest R&D and available trials. There is definitely more support to communities, and the optimisation of patient contribution and participation in research have proven to be very advantageous in other therapeutic areas.

The Future of Precision Medicine

Precision medicine and new-generation clinical trials are definitely here to stay. Oncology has led the way by setting the groundwork and has demonstrated that innovative translational research processes are possible. Multi-collaborative solutions, multiplex programs, academic and industry collaboration, and novel study designs have become preferred options over classic single sponsor, single disease type, and single drug trials.

About the author



Dr Aldo Poli has over 30 years' experience in the pharma industry, with a deep expertise in methodology and statistics. He is co-founder and CEO of OPIS, a full service CRO founded in 1998 that focuses on clinical trial management for multi-country Phase 1-4 trials, non-interventional studies, and medical device investigations.

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