… Senior industry experts advising young Biopharma Companies
And the Investor Community…

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Who are we?

We are a team of Biopharma Industry experts with medical and scientific training and qualification. Each of us has more than 30 years of industry careers including senior management and executive functions.

Our experience spans from basic research in Chemistry, Biochemistry and Immunology over Clinical Development, Regulatory Affairs, Drug Safety and Pharmacovigilance including Risk Management, Medical Affairs and Quality Assurance (GxP). With our expertise we provide integrated or focused strategic and operational advice and support to our clients. These include Biopharma Industry, Governmental Bodies and Investors.

What do we offer?

We offer expert advice and operational support for

- Due Diligence missions
- Feasibility and risk assessment of drug development projects for Investors and Analysts
- Organizational and Managerial advice for infrastructure development
- Interim Management and Board memberships
- Drug Development and Regulatory Strategy and Planning
- Focused Development and Regulatory advice tailored to the needs of young and start-up companies, including design and organization of Proof-of-Concept/Principle studies
- Preparation and conduct of Agency Meetings (FDA, EMA, national Competent Authorities)
- Design and implementation of Developmental Risk Management process
- Process definition and organizational design of Post-approval Drug Safety Risk Management

Pharmakon consultants are biopharma experts with a broad range of experience stretching from basic research through development, regulatory affairs, drug safety, pharmacovigilance including risk management, quality assurance and medical affairs.

They spent their professional career in small, mid-sized and big pharma. Having lived through creative and science driven enthusiasm and brilliant ideas of entrepreneurial personalities, they brought focus into processes and guided them towards pragmatic approaches for the translation of these great ideas into approved drugs.

They lead structural reorganizations focusing on simplified, targeted and more efficient processes

They have performed due diligence missions for the acquisition of products and companies based upon integrated assessments of all scientific and technical aspects of drugs under development.

Specialized Services

- *Due Diligence assessments of non-clinical and clinical drug developments*
    - *Assessment of drug development strategy, planning, execution and status*
    - *Suitability analysis of project and study data for regulatory purposes*
    - *Adequacy assessment of EMA and FDA targeted regulatory strategies*
    - *Strategic assessment of results from interactions with regulatory agencies and their impact on development programs*

- *Advice and support for meetings with potential licensees and licensors*

- *Assessment of improvement opportunities for development programs and regulatory strategies*

- *Interim management and Board membership*
    - *Strategic and operational support for the implementation of improvements in development, regulatory affairs, drug safety, quality management and medical affairs*

Due Diligence Assessments of Non-clinical and Clinical Drug Developments

Assessments of non-clinical and clinical drug development programs and the data intended to be established as well as data available already from studies performed and/or published in literature require integrated review of plans, programs and data with respect to several factors which are decisive for data acceptance by regulatory agencies for granting a Marketing Authorisation or an Approval (NDA, BLA). These factors include, but are not limited to, the chain from scientific rationale through targeted clinical indication and use.

It is not only the data per se originating from non-clinical, manufacturing and clinical work but also how they have been established to be credible and valid as well as whether they are representative and demonstrating with appropriate significance the effects to be shown.

Innovative and great scientific and clinical ideas frequently fail to result in Marketing Authorisations and Approvals or incur substantial delays because data, even scientifically or clinically convincing, have not been established in a way that provides to them the required quality and credibility (not been established following Regulatory Guidelines, Good Practice or Quality Systems requirements). It means that these data do not have the value required to support any claims for quality (manufacturing), safety (non-clinical) and efficacy (clinical).

Integrated assessment of these factors can only be performed based on the availability of appropriate expertise and experience with the assessor(s) or assessor teams.

"Pharmakon Experts have the expertise required for Scientific, Clinical, Regulatory and Quality Due Diligence assessments and have performed various Due Diligences for acquisitions of companies and drugs/drug candidates as well as for BioPharma companies as for Investors."

Assessment of Drug Development Strategy, Planning, Execution and Status

A data driven, well defined development strategy is the key to success in drug development. An innovative scientific idea which has been tested through pharmacological studies and eventually confirmed in pre-clinical disease models should drive the design of the development strategy.

Choices between potentially various indications and development objectives to achieve defined milestones have to be made early. Frequently, competing objectives like fastest route to proof of concept/principle, time to market or potential market size have to be assessed and decisions must be based on data, time and costs.

In many cases, development strategies are driven more by wishful thinking, rooted in a scientific concept, than supported by data. This may translate finally in clinical studies failing to demonstrate efficacy and safety of the developmental drug and result in loss of time and money.

A successful development strategy has to be implemented through well defined and integrated planning of manufacturing, pre-clinical and clinical activities. The complex interdependencies of internal, outsourced and regulatory activities offer plenty of opportunities to loose or to optimize time required to reach and completing each step in the development process.

Implementation and execution of the development activities should include considerations of time, resources and quality. The appropriate balance of this triangle is decisive for the created value of the development components. Data credibility and validity is the ultimate objective in order to establish a measurable value related to the investment.

Appropriate assessment of the development status of all development components as well as their impact on the timing of the overall program should be performed in a dynamic approach. Snapshots at critical time points alone do not allow for ongoing corrections of encountered problems and usually result in time delays. Analyses of root causes for delays and focused corrective actions provide possibilities to minimize the gaps between actual status and planned time lines. Without this type of information it is hardly possible to estimate potential delay in time, increase in costs and quality issues.

Suitability Analysis of Project and Study Data for Regulatory Purposes

Results and reports of studies in manufacturing, pre-clinical and clinical development may confirm expectations and satisfy by their existence formally the development plans. However, regulatory requirements and therefore the usefulness for regulatory purposes go far beyond the pure existence of the data.

Solid scientific rationale for protocol design, upfront defined criteria and procedures for the experiments and the evaluation of the data are basic to the creation of valid data and their reporting. As soon as clinical studies are being started, drug safety activities become an integral part of the development activities. For all of this, GxP quality requirements (cGMP, GLP, GCP) have to be followed in order to have finally credible, regulatory acceptable data and documents at hand which demonstrate data integrity, reliability and validity.

Quality requirement under GxP regulations include processes, procedures, qualified or validated equipment comprising as well IT hardware as software used for the production, processing and reporting of data. Proper documentation/audit trails from planning through completion and reporting of the study data are absolutely necessary to demonstrate GxP compliance.

The responsibility for compliance with these quality requirements lies fully with the Applicant/Sponsor of a regulatory submission and each of the performed studies. Regulatory agencies may inspect compliance with requirements. Failure to comply may result in regulatory actions from disqualification of data through suspension of further development. In cases that non-compliance is linked to humans suffering injury or death, civil or criminal court actions may be triggered.

Due to an increased safety concern and focus of regulatory agencies on data validity and acceptability, they are relying heavily on the mentioned and continuously tightening requirements. Data not established in compliance with regulatory requirements do not support any claims of efficacy and safety at any time point of development.

Adequacy Assessment of EMA and FDA Targeted Regulatory Strategies

Ideally, regulatory strategies should be designed in a way to address regulatory requirements for both, EMA and FDA. Despite harmonization of general requirements through ICH (International Conference for Harmonization) indication specific guidelines and clinical studies required for an EU Marketing Authorisation or a NDA or BLA approval by FDA may differ.

In order to include, at minimized efforts, requirements for EMA and FDA, a regulatory strategy should be designed with having not only both requirements in mind but also the flexibility of these authorities within their requirement frameworks.

Assessment of appropriateness of a regulatory strategy should be performed in preparation of any approach to a regulatory agency for a meeting or wriyyen submission concerning the acceptability of the development program.

For innovative drugs, novel regulatory strategies may be acceptable to the authorities if the underlying scientific rationale can stand the challenges of comprehensive scrutiny of the data to support quality, safety and efficacy of the drug in the claimed indication. The Pharmakon assessment identifies strengths, weaknesses and gaps in the strategy and advise on how to strengthen the argumentation and to fill the gaps.

Newer therapeutic approaches, such as cell-therapy and gene-therapy, require appropriate regulatory strategies. In many cases also regulators are on new territory and a well designed regulatory strategy is an instrument to drive agreements between the Applicant/Sponsor and the regulators. Therefore, the Pharmakon assessment will also identify strengths, weaknesses and gaps in the scientific rationale and in addition advise on shaping strategy and approach for meetings with regulators in these new fields.

For innovative drugs and new therapeutic approaches such assessments are highly advisable in order not to fail in EMA Scientific Advice Meetings (results binding for the Applicant but not binding for the CHMP) and FDA Meetings (binding for Sponsors and FDA).

Strategic Assessment of Results from Interactions with Regulatory Agencies and their Impact on Development Programs

Interactions with regulatory agencies always result in a written document in form of minutes, letters or formal answers to the questions asked. Experience shows that in many cases the interpretation of the content of the written documents is difficult or incomplete with respect to the impact on development programs. Even participants in agency meetings frequently fail to comprehend and remember all details of the discussions and by reading the document(s) received they tend to interpret their contents along their own ideas .

In addition, regulators usually respond only to the questions as asked and any not directly addressed issue may be left out of their considerations. This may result in major misunderstandings between Regulators and Applicants/Sponsors of the issue(s) at hand and misinterpretation of the reached agreements specifically on the side of the Applicant/Sponsor.

Critical examination of the outcome of these interactions with regulatory agencies offers a tool to identify not only aspects of misunderstandings and misinterpretations but also consider the impact of the results on parts of the development plan which are implicitly or directly linked to the responses/agreements. Early identification of issues raised and appropriate adaptation of the development program can save time and costs early in the process.

Advice and Support for Meetings with Potential Licensees and Licensors

Meetings with potential licensees require thorough preparation in order for the potential licensor to have the comprehensive understanding of the full potential of the developmental drug. Based on the agreements of the scope and contents of the meetings, appropriate material has to be prepared for pre-meeting information, presentations, discussions and data rooms.

Pharmakon provides advice and support for the preparation of scientific material embedded in the context of all relevant development information and regulatory strategy, linking to market perspectives and potential positioning. While providing full disclosure on all data it is important not getting lost in details and focusing on the decisive highlights, identifying important issues and their resolution.

Advice and support is important for the preparation and compilation of the scientific information in the data room which may include explanatory, specifically established documents providing easier access to the understanding of the scientific data, their context and implications.

Pharmakon provides support for meetings with potential licensees, licensors and investors by means of targeted presentations of scientific information and their interpretation in the light of their financial value related to regulatory and further development options which will broaden the perspectives and add objectivity. Active participation in discussions of the available scientific information and the positioning of their value for the further development and regulatory processes will add additional competences, expertise and long standing experience to the table.

Assessment of Improvement Opportunities for Development Programs and Regulatory Strategies

Innovative drugs offer the opportunity for novel development approaches. In most cases regulatory guidelines exist and provide the thinking of regulatory agencies about the requirements for the components of a development program based on their scientific considerations and the manufacturing, non-clinical and clinical standards at the time the guidelines were written.

For new therapeutic approaches, specific regulatory guidelines may not exist. In some cases, regulators are pro-active and publish drafts and concept papers based on currently available scientific information and their expected therapeutic applications. However, these documents are rarely very detailed and as a consequence the Applicant/Sponsor will have to design development programs with no or very limited guidance.

In both situations there are opportunities to design development programs and regulatory strategies in consideration of existing guidelines or regulatory principles and expectations. Tailoring of regulatory strategies may be applied to the properties of the drug, the targeted patient population and the "fine tuning" of the definition of the indication.

This means that there may be room for optimization of development programs and regulatory strategies with respect to the targeted development objectives while securing regulatory acceptability of the programs and strategies. Optimized development programs and regulatory strategies accepted by regulatory agencies increase substantially the value of the development projects for the owners of the drugs, potential licensees and investors.

Interim Management and Board Membership

Pharmakon consultants have managed in their industry careers various departments and organizations in small, mid-size and big BioPharma companies on national and international level. They also have been Board Members various companies during their organizational growth as well as their consolidation periods.

Pharmakon consultants have performed several interim management assignments, specifically in clinical development and regulatory affairs organizations of young and start-up companies as well as in well established organizations where transition periods had to be covered or organizational changes had to be designed and implemented.

Pharmakon consultants understand their role as Board Members in young and start-up companies in securing and increasing investment value through continuous support for, and sounding board and challenge to, the executive management, specifically in areas of their scientific, medical and management experience.

"Pharmakon consultants are qualified and have the experience to accept interim management assignments for, and board memberships as representatives of, investors."

Strategic and Operational Support for the Implementation of Improvements in Development, Regulatory Affairs, Drug Safety, Quality Management and Medical Affairs

There is no company where development, regulatory affairs, drug safety, quality management and medical affairs are optimized. Internal and external factors are changing rapidly and with young companies change is even faster. Continuous improvement is one of the most addressed aspects in all activities performed under pharmaceutical laws and regulations.

Investors usually do not have the time and/or qualified and experienced resources to assess and implement optimizations in organization and operations of companies.

Pharmakon consultants have assessed and implemented organizational and operational improvements in the R&D, Regulatory, Quality and Drug Safety areas of companies . Continuous improvement does not mean continuously ongoing changes of organization, processes and procedures. Change and innovation should be performed under consideration of overall costs and time due to potential disruptions of the ongoing activities/business. Usually, there are discrete events or moments in the development of a company where efficiency of organization and/or processes should be assessed and improvement actions implemented.

The assessment of improvement potential is the basis for proposing a strategy on what can and should be done in the setting of the company, its objectives, its financial and human resources, which activities are best kept in-house, should be out-sourced and what is required in-house in order to achieve optimal results through out-sourcing.

Pharmakon provides also support in the implementation of the strategy taking into account the internal opportunities and limitations for creating systems and processes which are conducive to continuous incremental improvement activities without unjustified disruptions of the business processes.

Biographies

Monique PODOOR, M.D.
President, Pharmakon S.A., Luxemburg

Monique is the founder of Pharmakon S.A., a consulting company providing strategic and operational advice, including due diligence, to Bio-pharma Industry, Trade Associations, Governmental Bodies and Investors.

Monique has been in pharmaceutical industry since 1977 and worked in both clinical research as well as regulatory affairs in positions of increasing responsibility to executive functions in several companies, among them Schering A.G., Beecham Pharma, Cyanamid International, Hoechst, Bristol-Myers Squibb, Schering Plough, and Janssen. Throughout her career she worked on a number of different developmental and marketed drugs, among others cytostatic drugs in breast cancer, hematological oncology, hormone therapy in prostate cancer, hormones for infertility and for contraception, anti-virals in HIV and hepatitis, antibiotics, cytokines, anxiolytics, antidepressants, antipsychotics, anti-Parkinson drugs, melatonin agonist, cardiovascular drugs, vaso-active drugs, gastro-intestinal drugs, topical corticosteroids, antihistamines, anti-hirsutism drugs, X-ray contrast media etc.

As a consultant Monique has been supporting the development and the subsequent filing of several new products through the different registration systems in Europe. She has acted as the key contact person with EMEA on several occasions.
She has been managing till November 2007 the operations of the EORTC, a pan-European academic research organization in cancer.

Monique is acting as a consultant to Trade Associations and Health Authorities on the implementation of the EU Clinical Trial Directive and specific clinical research topics. She is a regular lecturer at the University of Brussels and on several international platforms and the co-author of the reference manual “Clinical Trials in Belgium”.

Gerd JOHNSCHER, Ph.D.
Senior Consultant, Pharmakon S.A., Luxemburg

In January 2008 Gerd joined Pharmakon S.A. as Senior Consultant. Prior to this he was with UCB S.A., Belgium. There he was for 5 years Head of CNS Development where he developed Keppra, a blockbuster anti-epileptic drug. Gerd created and managed over 7 years a global Regulatory Affairs organization which performed UCB's first and successful FDA NDA and EMEA centralized submissions and approvals as well as the first Mutual Recognition procedure. He built and headed for 10 years global, integrated Drug Safety (development and pharmacovigilance) and Quality Assurance (GxP) organizations. He also developed drug safety Risk Management processes and procedures. In parallel he managed for 7 years Global Medical Affairs and for 3 years HS&E. He also conducted various due diligence assessments for acquisitions of licenses and companies. His last position with UCB was Senior VP, Advisor to the CEO. He was also Board Member of various companies.

Prior to joining UCB, Gerd spent more than 2 years with G. H. Besselaer Assoc. (now Covance) in charge of Business Development Europe, including regulatory and development consulting for US biotech and Japanese companies.

He started his industry career with Hoechst AG, Germany working for 5 years in Biochemical Research about T-cell immunology and lymphokines (2 patents) as well as carbohydrate chemistry, which resulted in an assignment by Pharma Production for the transfer of a manufacturing process from Canada to Germany. He built the basis for his development and regulatory career during 8 years in International Clinical Research and Regulatory Affairs with the responsibility for Northern Europe and North America. He finally spent 2 years in the Strategic Planning Group of the Pharmaceutical Division in charge of international development and regulatory strategies.