

# Yao-Chang Xu - Founder & CEO, Abbisko

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*After a Big Pharma career in the US, Dr Yao-Chang Xu returned to China and in 2016 founded Abbisko, a biotech that began with a focus on small molecule oncology treatments. He shares the company's evolution over the past eight years, including its exploration of broader therapeutic areas, the major milestones Abbisko has reached with its two lead candidates, and its USD 70 million upfront collaboration with Merck for one of these therapies, pimcotinib.*

**Could you describe the vision you had when founding Abbisko, particularly considering your background at Novartis and the biotech landscape in China at the time?**

When I founded Abbisko my vision was clear: to establish a world-class biotech company dedicated to discovering novel medicines, particularly those that address underserved medical needs in China's healthcare landscape. At the time, there was a significant gap in medical technology and drug discovery research in China, and the majority of local pharmaceutical companies were only interested in generic medicines. Our aim was to discover innovative, best-in-class and first-in-class drugs using precision medicine and immuno-oncology, approaches that represented a new generation of drug research and which had seen technological validation.

We hope to address local unmet medical needs and contribute to the global healthcare ecosystem with our innovative treatments. Disease areas such as liver and gastric cancer, which are very prevalent in China and Asia more broadly, carry limited global research focus, and therefore

underscore the urgency and potential impact of our mission.

**Building a world-class biotech company in China is a lofty goal. Given the less mature local market compared to the US, how did you approach establishing Abbisko under these circumstances?**

It required careful consideration. My experience at Novartis, particularly with starting up the Novartis research centre in China, was pivotal. Over the span of five years (2007-2012), we started from scratch and built a world-class drug discovery research team that embarked on multiple innovative projects, showcasing China's impressive talent pool and which drew both local and international attention. The supportive environment from local and central governments, coupled with the growing societal view that the pharmaceutical industry is integral to the well-being of the country, also provided that conducive atmosphere. Our mission is to save lives, so we are proud of what we do! Of course, the influx of major pharmaceutical companies like Merck, Lilly, and Novartis further elevated interest and expertise in the pharmaceutical sector in China. The dynamic between China's burgeoning economy, regulatory bodies, and perception that biotech brings about social good, helped to nurture talent and provide that fertile ground necessary for Abbisko to thrive. Abbisko was able to leverage skilled professionals who had honed their expertise in multinational settings and were looking to create positive impact.

**Why did Abbisko decide to focus on small molecules in an era where biologics and complex modalities dominate the pharmaceutical landscape?**

The decision to focus on small molecules stems from both tradition and the modality's strategic advantages. Traditionally, small molecules have been at the forefront of drug discovery research due to their versatility and established research methodology, with applications spanning oncology, cardiovascular and metabolic disease, antivirals, and neuroscience. While biologics and advanced cellular therapies such as CAR-Ts have gained more attention over the past few decades, our founding team's deep and extensive experience is primarily with small molecule drug discovery and this naturally played a critical role. Our founding team has been principally involved in numerous small molecule projects that have achieved regulatory approval and commercial success. Further, we have observed that despite the rise in popularity of other modalities, small molecules consistently receive a greater number of FDA approvals annually, underscoring their enduring relevance in drug development. Historical data and therapeutic advantages support our

belief that small molecules will continue to offer significantly differentiated opportunities in drug innovation.

Moreover, focusing on small molecules allows us to leverage existing technology and capitalize on our team's proficiency in this area.

There is sometimes the misconception that small molecule drugs are easier to discover due to the modality having been around for longer than others, but this perception is flawed. In reality, small molecule discovery presents unique challenges, particularly in early stages of discovery because it requires thoughtful R&D experience. Whereas antibodies can be designed by using different cellular or animal-based environments as a starting point, small molecules require careful construction and identification at every step such that the molecule can bind precisely to a specific target while maintaining its desired therapeutic effect. This involves a time-consuming exploration of a vast array of chemical structures and configurations, where the shape and properties of the molecule play an important role. A strong small molecule candidate can provide unique clinical development and manufacturing advantages, as well as improved clinical outcomes.

**Abbisko focused on oncology as its primary therapeutic area. Can you elaborate on how your clinical development strategy has evolved over time?**

When Abbisko was founded, our primary focus was indeed on small molecule oncology treatments. That strategic decision was driven by the need for focused expertise, alongside investor attention and support for such types of biotech start-ups. Over the past five to eight years, however, our approach has evolved organically.

While our foundational work remains centred on oncology, we have encountered exciting opportunities where our research can extend beyond this initial scope. One notable example is our FGFR2/3 inhibitor which was originally designed to treat solid tumours. Through ongoing research, we have discovered its potential application in a condition called achondroplasia—a genetic disorder affecting bone growth in children, resulting in short stature. This expansion into achondroplasia exemplifies our commitment to exploring broader therapeutic areas. Although still in early stages, we have observed promising developments. Our molecule has the unique feature of higher selectivity against the FGFR1 target. By avoiding FGFR1-associated toxicities, this can potentially offer a better safety window and efficacy in paediatric patients.

This strategic pivot demonstrates our agility and commitment to maximizing the impact of our scientific discoveries across multiple therapeutic areas. While oncology remains our cornerstone, our ongoing research may uncover applications that benefit patients beyond cancer.

**Your clinical development strategy spans multiple regions. Can you elaborate on the sequencing of your clinical trials? Have you typically started in China and then expanded globally, or has it been more parallel?**

Our clinical development strategy is meticulously planned to suit each project's unique requirements. For example, with our first molecule, pimicotinib, which targets rare disease with CSF-1 inhibition, we initiated clinical studies in the US where clinical expertise was essential. We then transitioned to a phase Ib, and strategically chose China for its advantageous patient recruitment and local clinical operations expertise. We have now progressed to a phase III study with trial sites across China, USA, Canada, and multiple European countries. This global strategy not only ensures robust clinical validation across different patients but also facilitates global regulatory acceptance.

This multi-regional phased approach has been instrumental in allowing us to validate early efficacy and observe safety signals across different patient populations, optimizing our clinical pathway. The positive results from our China-based trials for pimicotinib also enabled us to gain US FDA and China NMPA Breakthrough Therapy Designation, as well as EMA PRIME designation. Such accomplishments underscore the effectiveness of our approach in expediting our drug candidates' regulatory pathway while also optimizing development resources.

**Looking at Abbisko's pipeline, you have a diverse array of assets. Could you highlight which ones are particularly exciting for you right now? Given your goal to establish Abbisko as a global biotech player, which projects do you see as pivotal in this journey?**

Each project within our pipeline holds significant potential, and there are two in particular with crucial milestones that I will highlight.

Firstly, ABSK011, also known as irpagratinib, is an FGFR4 inhibitor that has shown compelling proof-of-concept data. Last year, we reported an impressive 40.7 percent objective response rate (ORR) in pre-treated liver cancer patients, surpassing current therapies in terms of response rate. This achievement may enable us to initiate a registrational clinical trial, which is currently in ongoing

discussions with regulatory authorities. This advancement is pivotal as we aim to bring a potentially transformative treatment for liver cancer, which affects hundreds of thousands of people worldwide, closer to market for patients.

Secondly, ABSK021, or pimicotinib, has also delivered extremely strong results. Initially targeting TGCT (tenosynovial giant cell tumor), pimicotinib demonstrated a remarkable 68 percent ORR at week 26 – the highest rate of response observed with a medication for TGCT in a proof-of-concept study. This outcome once again underscores our commitment and leadership in addressing unmet medical needs. Now in a phase III study, enrolment for this trial has already been completed and we expect top-line data by the end of the year. Should the results be positive, we look forward to promptly submitting a New Drug Application (NDA), a critical step towards bringing this innovative therapy to patients.

These developments truly demonstrate our commitment to addressing unmet medical needs but also highlight our capability to secure regulatory endorsements such as Breakthrough Therapy Designation in the US, Europe, and China. As we navigate these critical catalysts, 2024 promises to be a transformational year for Abbisko, solidifying our position in the global biotech landscape with potentially breakthrough treatments on the horizon.

**Are you exclusively focused on developing best-in-class or first-in-class medicines, or are you open to pursuing programmes that may not achieve these distinctions based on your data?**

Our primary objective at Abbisko is to discover and develop medicines that either lead as best-in-class or pioneer as first-in-class therapies. Currently, the majority of our programmes align with best-in-class aspirations, aiming to set new standards in their respective therapeutic areas. For instance, our efforts with pimicotinib exemplify our pursuit of best-in-class status, demonstrating superior efficacy in treating TGCT.

With irpagratinib for liver cancer, we have positioned ourselves at the forefront of this field. Former contenders in this space have discontinued their efforts, leaving us as the leader in advancing a potentially novel first-in-class treatment option for liver cancer.

**It seems Abbisko has established significant partnerships, with Merck being prominently involved. Could you elaborate on whether this collaboration model**

**represents the future strategy for Abbisko, or is it a current necessity to advance your medical developments?**

Our collaboration with Merck indeed plays a crucial role in our current strategy. This partnership stems from the recognition of the promising preclinical and clinical data we have generated with pimicotinib, resulting in USD 70 million upfront, significant milestone payments and double-digit royalties. Discussions with Merck have initially centred on licensing in commercial rights for China. As we progress towards potential NDA filing by the end of the year, we open up the possibility to engage in global expansion.

For a young biotech company like Abbisko, navigating the complexities of commercialization presents challenges. While we have the expertise and resources necessary to conduct research and clinical trials, building and scaling a global commercial infrastructure requires extensive capabilities and investments. Partnering with a multinational like Merck not only accelerates our ability to bring transformative therapies to market swiftly but also leverages their established global presence to maximize the commercial potential of pimicotinib.

As we plan for the future, we are evaluating each product on a case-by-case basis. For instance, with our second asset focused on liver cancer, a critical therapeutic area for us, we are leaning towards commercializing this asset ourselves in China.

**Regarding your partnerships with AstraZeneca and Lilly, could you clarify their contributions—are they financial or strategic, such as providing expertise or resources? Also, how do these collaborations align with Abbisko’s future strategies?**

These partnerships help to validate Abbisko’s discovery engine and allow us to leverage the strengths and resources of established players in the pharmaceutical industry. Such collaborations extend far beyond financial investments. They involve significant transfer of scientific knowledge and expertise. When we engage with partners like Merck, Lilly, or AstraZeneca, we also gain access to their extensive research and development histories for certain therapeutic targets. This includes insights into previous studies, technological advancements, and strategic approaches that they have employed. Our scientists and clinicians can then leverage this foundation to refine and tailor molecules to meet various criteria for clinical trials. Essentially, it is a collaborative effort where we absorb their previously explored methodologies, and then we integrate our capabilities and insights to drive innovation forward.

**What steps do you believe are necessary for Chinese companies like Abbisko to achieve global recognition comparable to longstanding giants such as Amgen or Gilead?**

Having worked extensively with industry leaders including Lilly and Novartis, I have witnessed first-hand the operational scale and strategic depth of Big Pharma. Transitioning from that environment to founding Abbisko was driven by a vision grounded in ambition and innovation.

In biotech, success hinges on meticulous groundwork. Unlike large pharmaceutical conglomerates that manage diverse portfolios, biotech companies often concentrate their efforts on select projects and special areas. At Abbisko, we emphasize starting with a strong scientific foundation, focusing on innovative targets that address critical unmet medical needs. We aim to bring our first and subsequent programmes to market, where successful performance can validate our vision for continued innovation and attract further investment. This process builds our capabilities and positions us for sustained growth in the industry.

Ultimately, our goal at Abbisko is to establish a robust presence on the global stage, marked by transformative healthcare solutions that resonate worldwide.

**What are some of the key trends within the financial market impacting Abbisko?**

Our Initial Public Offering (IPO) in September 2021 coincided with a pivotal turning point in the global biotech market where the landscape saw a substantial slowdown in biotech investment and market support. As a result, we have been navigating such challenges. The current market pressure, particularly in regions such as Hong Kong where Abbisko is publicly listed, has led to negative price impacts and lower trading volumes for HK-listed biotech companies. I believe this is partly due to natural market cycles, though this downturn seems to be lasting longer than usual comparatively.

The concern lies in how a weak stock market impacts our ability to raise funds for our ongoing programmes. Fortunately, Abbisko remains financially strong. We currently have over USD 300 million in cash reserves, enabling operational runway for multiple years. In addition, we expect to see cash flow from our partnerships over the near-term. It is crucial that we continue focusing on delivering strong performance and maintaining operational excellence. Despite market challenges, we remain committed to our strategic objectives and believe that our efforts will be recognized when market conditions improve. Our resilience lies in our ability to adapt, innovate, and

communicate effectively with all stakeholders, ensuring they understand our value proposition and the positive impact our endeavours carry.

**How did Abbisko manage to maintain such high ownership stakes despite engaging in partnerships? Was this a deliberate strategy?**

Maintaining substantial ownership was intentional for us. We have been disciplined in managing our resources carefully, drawing upon our experience working at both large and local pharmaceutical companies. This approach ensures that we retain significant control and leverage even as we collaborate with partners. We must be well-prepared to progress our pipeline without solely depending on external funding.

**What would you like our readers to take away about Abbisko?**

Abbisko is a remarkable company dedicated to improving lives. We have a very clear vision and an experienced team in managing drug discovery research and development, as well as company operations. We are strongly positioned, ready to advance multiple projects into later-stages. I believe we will soon see even greater progress and achievements.

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