



A LIFETIME OF CANCER RESEARCH, A HISTORIC PATENT DEAL

BY KAREN FERRICK-ROMAN

*University's Largest
Licensing Agreement—
with Alumnus—
Puts Anti-Cancer
Compounds on Path to
Commercialization*

The largest licensing agreement in Duquesne University's history brings the fruits of Dr. Aleem Gangjee's career-long fight against cancer all the closer to commercialization.

"This is a unique opportunity for one of our researchers to join forces with an alumnus who can further his research efforts and attempt to bring these compounds to market," says Pharmacy Dean J. Douglas Bricker.

The November agreement with FLAG Therapeutics cements Duquesne's reputation as a top-tier research institution, strengthens the relationship between Duquesne and the company's President and CEO Dr. Frank Sorgi, a graduate of the School of Pharmacy and

Dr. Aleem Gangjee, holder of the Adrian Van Kaam, C.S.Sp., Endowed Chair for Scholarly Excellence, enjoys the opportunity to support graduate assistants, who participate in research efforts in the wet lab.



the School of Business—and buoys the lifelong dream of researcher Dr. Aleem Gangjee to fight back against the villainous disease that claimed his grandmother's life and the lives of about 7.6 million people every year.

It's hard to pick the sweetest part of the deal.

Living the Researcher's Dream

The licensing agreement extending beyond 2035 encompasses the career portfolio—nearly 50 patents—of pharmacy professor Dr. Aleem Gangjee. Growing up in India, Gangjee planned to be an industrial chemist—until his grandmother's breast cancer diagnosis. Her struggle shaped Gangjee's professional life, moving him into medicinal chemistry and a career that would demand every ounce of his creativity and scientific shrewdness.

Arriving at Duquesne in 1979, Gangjee started a lab with borrowed glassware and a \$10,000 United Way grant. In 2013, he achieved the near-mythic status of holding five concurrent National Institutes of Health grants; his proposals have attracted more than \$18.2 million to Duquesne.

The funding has not only provided the means to buy glassware, equipment and chemicals, but to support and train the next generation of researchers, to subcontract with collaborators, and to design computer-generated molecules that physically and chemically thwart cancer cells. A Distinguished Professor in the Graduate School of Pharmaceutical Sciences, Gangjee has created a think tank in his Mellon Hall office, with photos of his beloved grandchildren always watching. But the space is dominated by carefully choreographed stacks of student work, articles he is writing, articles he is reviewing, data from collaborators and discoveries in the field. One wall holds some patents and other awards, like the 2012 American Association of Pharmaceutical Scientists Research Achievement Award in Drug Discovery and Development Interface.

This award recognizes Gangjee's latest compounds, linchpins in the licensing deal: two families of water-soluble drugs (potentially allowing them to be administered orally) designed to target and destroy cancer cells in at least two different ways. The first class

of compounds ever to combine antiangiogenic and antitubulin activities into one molecule takes advantage of the narrow window of time when the extraordinary blood supply that supports aggressive tumor growth can be suppressed by pharmaceuticals. With the blood supply diminished by the antiangiogenic effect and the cancer cells already weakened, antitubulin compounds further inhibit cancer cell growth.

The second family, formed of folate-targeted anti-cancer compounds, binds selectively to receptor sites found predominately on cancer cells and interrupts cell multiplication. By physically and chemically blocking tumor growth, these compounds kill tumor cells—and target only tumor cells, leaving normal cells unharmed. This selective therapy is less likely to sicken cancer patients with side effects.

Both of these novel approaches trick cancer cells, acting like Trojan horses carrying the compounds inside the tumors to pack their deadly double punch. Preclinical studies suggest that the compounds could be used to treat multiple types of cancer, including some ovarian, breast, lung, brain and pancreatic cancers—and surpass conventional therapies in their safety and efficacy profiles.

The compounds show two other remarkable advantages: the agents are not susceptible to drug resistance and are particularly promising in both late and early stages of the disease.

"Dr. Gangjee is a highly respected researcher and FLAG is honored to have the opportunity to advance his discoveries from the bench into the clinic—and ultimately to individuals in need of new therapeutic cancer treatment options," says Sorgi. "We already have identified lead clinical candidates, each supported with encouraging comparative in vivo data versus current therapies, and we look forward to advancing these compounds into clinical trials."

Recognizing the Beauty of the Promise

The pharmacy school asked Sorgi to take a look at Gangjee's patent portfolio. A member of the Dean's Advisory Council with an entrepreneurial science career spanning more than 20 years in early development and



Dr. Aleem Gangjee and Dr. Frank Sorgi (above) enter a new partnership as Sorgi launches a company around Gangjee's compounds.

commercialization of compounds in the U.S. and abroad, Sorgi had experience in all steps of the drug development process, from drug inception, preclinical and clinical development, to manufacturing and commercialization. He agreed to develop a business plan to license the patent portfolio of his former professor and to help find someone to execute it.

Even though he'd worked for many startups, he wasn't expecting to start his own company. He'd known Gangjee for years—in fact, Gangjee, then department chair of

the pharmacy's graduate school, presented Sorgi with his master's hood in 1990. Sorgi certainly was aware of Gangjee's anti-cancer research. "But the more I learned about the work, the more intrigued I was," says Sorgi. "The small compounds were very intelligently designed and the strong patent portfolio around the work was incredibly impressive."

In June 2013, Sorgi became founding president and CEO of FLAG Therapeutics Inc., creating a company around Gangjee's portfolio and making his relationship with Duquesne stronger than ever.

Duquesne had provided Sorgi's academic foundation for three of his four degrees: pharmacy ('87, '90) and an MBA in management ('90) from the School of Business, where son Jacob is a student. The pharmacy and MBA degrees were unusual combinations at the time, and Sorgi discovered a love for the business of science. His Duquesne training, topped by a Ph.D. in pharmaceutical sciences from the University of Pittsburgh, set the stage for a varied career: as vice president of global science and technology at Patheon Pharmaceuticals, responsible for all early development from inception through safety, dosage and efficacy evaluations; and as vice president of research and development at DPT Laboratories Ltd., growing the company into a leading contractor for liquid and semi-solid pharmaceuticals. He expanded his commercial skills and expertise as program director at BioMarin Pharmaceutical, overseeing a small molecule drug for pediatric asthma and a joint venture for a compound to treat a rare genetic disease; worked at a number of startups; and served as CEO at OPTIME Therapeutics, focusing on liposome drug delivery for an array of disease indications.

FLAG Therapeutics, based in the biotech/pharmaceutical industry hub of Raleigh, N.C., is the first company Sorgi has founded, but is more than a business opportunity. "Given

my long-standing relationship with Duquesne, I feel that I have a duty, an obligation to do good by this opportunity," says Sorgi. "Drug development is a long, bumpy road that requires a tremendous amount of patience, dedication and know-how. It could be up to 2 ½ years before FLAG can advance an investigational compound through the first phases of development. Ultimately, the goal is to get these compounds into the hands of people who can benefit from new treatment options."

The Bigger Picture Behind the Big Deal

A deal with a company like FLAG, whose sole focus is Gangjee's compounds, offers the best opportunity for the compounds to enter the marketplace, says Dr. Alan W. Seadler, associate provost for research and technology. Plus, the pervasive Duquesne thread woven between Gangjee and Sorgi provides a special connection.

"This licensing agreement for a major anti-cancer agent portfolio is to a company that we feel can take these compounds and develop them into future therapeutics that could benefit many cancer victims," says Seadler. "We are particularly pleased to be working with a graduate from our pharmacy school."

While this licensing agreement is historically significant, it also represents Duquesne's rising reputation among research institutions.

"I think we have always had a focus on technology transfer, making University-based research accessible and available to the marketplace, but some things this year have really helped to get Duquesne moving," says Seadler. The University holds 95 active patents, had three spin-offs showcased at a recent AlphaLab Demo Day and signed off on a licensing deal for Dr. Patrick Juola, associate professor of computer science, who drew international attention when his technology identified J.K. Rowling as the actual author of a crime fiction book.

Moving research from the scientist's bench to bedside aligns perfectly with the University's mission. "Research is part of our higher education commitment, and having research work for community development, to create jobs and to improve the lives of others is in keeping with the University's mission of service," says Seadler.

For Gangjee and Sorgi, who want to offer more hope in their compassionate answers to queries from desperate physicians and parents of sick children, the best may be yet to come.

"We pursue the discovery of novel drugs because of the challenge to stop this dreaded disease in its tracks," says Gangjee. "To be able to partner with a company that affords the promise that these discoveries will have—the opportunity to be used in patients who need them—is a lifelong dream." ■