

**Offering the Most Comprehensive and Most Clinically Informative Genetic Test on the Market for Individuals with Autism and other Disorders of Childhood Development, Lineagen, Inc. is Well Positioned for Future Growth**

**Biotechnology  
Diagnostics  
(Private)**

**Lineagen, Inc.  
423 Wakara Way, Suite 200.  
Salt Lake City, Utah 84108  
801-931-6200.  
www.lineagen.com**



**Michael S. Paul, Ph.D.  
President and CEO**

**BIO:**

Dr. Paul is a co-founder and has served as President and Chief Executive Officer of Lineagen since its inception. Dr. Paul has been recognized as one of Utah's leading venture entrepreneurs and was a Utah Governor's Appointee to the Steering Committee for the Utah Technology Industry Commission. Prior to Lineagen, he was President and Chief Operating Officer of Lineagen Research Corporation, Vice President of Business Development at Huntsman

Biotechnology Corporation and Director of Strategic Development at NPS Pharmaceuticals.

Dr. Paul has also worked at the University of Utah Technology Transfer Office where he participated in the marketing of University inventions to the pharmaceutical and biotechnology industries. Dr. Paul is a licensed patent agent of the United States Patent and Trademark Office. Dr. Paul earned his B.A. from Colby College and his Ph.D. in Molecular Biology and Biochemistry from the University of Utah.

**Company Profile:**

Lineagen's mission is to accelerate and enhance the diagnostic evaluation of complex medical conditions so that the best possible outcomes can be achieved for patients and their families. Our first commercial offering, FirstStep<sup>DX</sup>, provides physicians with a fully integrated genetic testing, genetic counseling, and developmental screening service to aid in the diagnostic evaluation of individuals with Autism Spectrum Disorders (ASD) and other disorders of childhood development. In addition to our deep commitment to ASD and developmental delay, we have ongoing scientific programs in the areas of multiple sclerosis (MS) and chronic obstructive pulmonary disease (COPD).

**Interview conducted by:  
Lynn Fosse, Senior Editor  
CEOCFO Magazine**

**CEOCFO:** Dr. Paul, what was your vision when you cofounded Lineagen and what are you doing today?

**Dr. Paul:** Since its inception, Lineagen has been focused on the value of genetics in understanding at a molecular level the basis of complex diseases. We started with ASD and other disorders of childhood development because they are highly genetic in origin and because they are difficult to diagnose, as their clinical symptoms are non-specific. The Lineagen vision is to enhance and to accelerate the diagnostic evaluation of these and other complex disorders so that best outcomes can be achieved for the patients and their families.

**CEOCFO:** Is it widely believed that you can do genetic testing for autism?

**Dr. Paul:** Many people know that the prevalence of autism has risen dramatically in our society, in fact the whole world. Estimates are that up to 80% of the cause of autism could be genetic in origin. For example, many people know that twins can both share autistic traits. Genetic testing is now recommended by the American College of Medical Genetics as a first-line genetic test for all individuals with ASD but also for individuals with apparently non-syndromic developmental delay and other conditions such as intellectual disability. In aggregate, these disorders are estimated to affect over 500,000 kids per year. While the public sector may not fully understand the importance of genetic testing, the medical community is well aware and in fact, a recent article out of Stanford University indicated that too many children with autism miss genetic evaluations, and missing a genetic factor that is associated with autism could have dire consequences. Awareness is very critical, so there needs to be increased

awareness by the public, but there is a definite understanding within the medical community.

**CEOFO:** What have you discovered at Lineagen which others have not?

**Dr. Paul:** Through years of genetic research partnership with the University of Utah and through our more recent partnership with The Children's Hospital in Philadelphia, Lineagen has invested millions of dollars of its own capital to discover many more genetic factors than were previously known to be associated with autism. With The Children's Hospital in Philadelphia and University of Utah, we have recently completed one of the largest genetic validation study ever performed in autism to confirm the clinical importance of these novel genetic factors as well as factors previously published in the medical literature. As a result, we have just recently brought to market a new customized whole-genome genetic test integrated with genetic counseling, which will increase the clinical detection of genetic variants associated with autism by more than two-fold over all existing tests on the market. The reason why this is important is that a recent clinical study out of the Children's Hospital in Boston showed that detecting a genetic variant associated with autism or other disorders of childhood development can have a significant and direct influence on that patient's clinical management. These management changes may prevent the need for other types of clinical procedures such as MRIs, and could get the patient into more directed and appropriate treatment regimen including language treatment as well as certain drug treatments.

**CEOFO:** How does the testing actually work?

**Dr. Paul:** We actually take a sample of a child's cheek cells, not a blood sample. We have a twelve-parent advisory committee that has informed us that many kids with autism have to be anesthetized for simple procedures such as going to dentist or getting their hair cut, so taking a blood draw from a child with autism is a very

traumatic experience. Therefore, Lineagen was the first to bring to market a cheek swab in recognition of the patient's needs. The cheek swab is sent to Lineagen and we look across the entirety of that child's genomic makeup. We identify the differences in letters or sentences within the regions of the DNA that makeup a large bulk of your genetic information. We can identify where the sentence or the letters or DNA words have changed in a child with autism, versus in an individual without autism. We then can tell that doctor and the parent what the changes might mean for the family and whether the family has increased risk of having a second child with the same genetic variation. We can also inform the doctor what clinical changes might be most appropriate for that child with this particular change in their genetic information. It is a whole-genome test

**While there are non "no-risk" deals in the marketplace, we believe Lineagen provides a unique, risk-mitigated investment profile, a great syndicate of investors, a great management team, and an IP barrier to entry to the most comprehensive and most clinically informative genetic test on the market for individuals with autism and other disorders of childhood development. - Michael S. Paul, Ph.D.**

coupled with very personalized reporting as to what is specific for that individual patient.

**CEOFO:** Are you able to test potential parents to determine who may be at risk to have a child more likely to have autism?

**Dr. Paul:** We know that these genetic changes can be both inherited, in that they were passed on parents, but a more recent scientific discovery is that there are many genetic variations that are in fact not passed on from the parent, but rather happen *de novo*, meaning that these changes happen during the child's development. In fact, a recent study out of Iceland has shown that these types of *de novo* changes happened more frequently in children who have older dads at conception. Therefore, the age of the dad at conception has now been shown to be a "risk factor" for having a child with autism. This should not be en-

tirely surprising as we have all known that the mother's age at conception is a higher risk factor for other types of developmental disorders including Down Syndrome. But, this recent finding may indicate why there might be an additional factor contributing to the rise in the prevalence of autism in terms of the age of the dad at the time the child was conceived. What many people do not know is there is a treatment or intervention for autism. While not a drug treatment per se, although there are many pharmaceutical companies with autism drug development programs, there is a clinically effective behavioral treatment with clinical effectiveness demonstrated in randomized controlled clinical studies. These rigorous scientific studies are just like a drug studies and have shown to early intensive behavioral intervention to be effective in kids as young as eighteen months, yet

the average age of diagnosis remains around six years of age. We know that if intervention occurs early, many children can live more independent lives and may have sustained gains in their IQ. The desperate situation is that it takes longer than necessary to get a comprehensive diagnostic evaluation, and where Lineagen

steps in is to enhance and accelerate the diagnostic evaluation so that these children can receive appropriate intervention as soon as is practicable.

**CEOFO:** Have the appropriate parts of the medical community been paying attention to Lineagen?

**Dr. Paul:** One of the key milestones for Lineagen is to inform the medical community of our science and test through peer-reviewed publications. To that end, we expect publication of our validation research in the fourth quarter of this year. This manuscript has already been submitted for publication to a peer-reviewed scientific journal, and is under review. This will help us disseminate the valuable information about these discoveries made in collaboration with the Children's Hospital of Philadelphia and the University of Utah, and will show an increase the clinical detection of

genetic variance associated with autism. We will be able to reach more clinicians, more families, and more people who can benefit from this important and clinically actionable information.

**CEO CFO:** Are autism organizations paying attention?

**Dr. Paul:** There are many opinions in the broader autism community as to the causes of autism, primarily because much less is known about the causes of autism compared to what is known about the cause of the breast cancer or causes of other complex disorders. On a population-wide level, it has been shown in numerous studies that vaccines are not causative for autism. I am not stating that there are no environmental components, and I am not stating that vaccines do not affect every person differently. I am stating that there are certainly environmental influences to autism, just like there are environmental components to all complex disorders. For example, there is a company here in Utah called Myriad Genetics, which has discovered genetic variances that increase the risk for breast and ovarian cancer. Just harboring the genetic variants does not mean you get cancer, it means that you are more susceptible. There will be environmental components that are yet unknown for autism. There are also still genetic factors that are yet unknown. I think we are beginning the age of increased knowledge about these causative factors and we all know that autism is a spectrum of disorders. There are individuals with autism spectrum disorders that are higher functioning in terms of language and there are others challenged with language development. There may be different genetic factors and different environmental factors that have influenced this spectrum of disorders in that particular individual. At the end of the day, it is very individual and each individual with autism is different. There are a number of genetic variations found in individuals of autism but there is still much to learn. The opinions in the broader autism community vary just because we do not know as much as a scientific and medical community as we do about other conditions.

**CEO CFO:** Is Lineagen working in others arenas as well?

**Dr. Paul:** Our initial commercial offering is an integrated genetic testing and counseling service called FirstStepDx. We named it that because parents tell us that they are on a “diagnostic odyssey” and we want to help them along their journey. There is a very similar situation for MS, which is a neurological disorder that Lineagen is working in which the diagnostic process is lengthy. Multiple sclerosis is not diagnosed until there have been a number of neurological attacks. The diagnosis is primarily by MRI detection of lesions on the brain, brain stem or neurological tissues, after the attack has happened. We are working with clinicians at the University of Utah to discover and bring to market a better diagnostic assay that might predict which type of disease course a person is experiencing. We have partnered with an organization called Fast Forward, a wholly-owned subsidiary of the National MS Society to advance our program. Fast Forward carefully reviewed our program and they evaluated it with a number of specialists, both in the business and MS community, and determined that our program is very scientifically important as well as a valid approach. They have partnered with us to fund the further development of our MS and diagnostic assay. Those are two primary product opportunities, both aimed at accelerating and enhancing the diagnostic evaluation process of a neuropsychiatric disorder in autism and a neurological disorder in MS. We also have a program in a respiratory disorder that we are looking to help drug companies develop better drugs for chronic obstructive pulmonary disorder.

**CEO CFO:** How far can Lineagen go with the funding you have in place?

**Dr. Paul:** We have been very efficient with our current funding. We are one of the few diagnostic companies that have achieved a market presence and generating revenue on less than \$11 million of initial equity. We are currently raising \$20 million Series-B growth capital round. We have secured \$10 million of that from our current Series-A investors, as well as from new investors. We are seeking

\$10 million in new equity commitments, to achieve profitability. We need the capital to grow our commercial franchise as Lineagen has sold the FirstStepDx test in over thirty states across the country and to over three hundred physicians. The Series-A investors are very committed and still have additional reserve capital in their funds for Lineagen. Our goal is to close this round as soon as is possible so that we can get on with building the business.

**CEO CFO:** Will the test be reimbursable at some point and does it make a difference?

**Dr. Paul:** It does make a difference. The reason why it makes a difference is it improves clinical management of patients. It is important to know that many insurance companies including some segments of Blue Cross/Blue Shield and others have issued favorable coverage policies on the type of technology that Lineagen uses, not on Lineagen test specifically, but the type of technology that our test as well as competitor tests use. To date, we have submitted claims to over 150 insurance companies and we have been paid by a number of these carriers. Not only is our test reimbursable today, with the publication of our peer-reviewed manuscript, it will be twice as informative as competitive tests. We believe that reimbursement environment will only become more favorable because of the value that the test delivers.

**CEO CFO:** Why does Lineagen stand out to investors and people in the biotech community?

**Dr. Paul:** We stand out because we have identified a solution for a unique unmet need in a very important area. With autism, the reason we stand out is you do not need a new drug to change a life, these individuals need a more comprehensively diagnostic evaluation sooner, so that these genetic variations are identified and appropriate clinical management can be implemented much sooner than the current average of diagnosis, which is about 5-6 years of age. As an investment opportunity, we stand out because we have a unique opportunity profile. This is projected to be our last round of capital before we get to prof-

itability. We have an IP differentiated product and in the diagnostic sector, IP differentiated companies such as Clarion Diagnostics are being purchased by larger companies, whether those companies are large laboratory or large healthcare companies, for six to seven times their trailing twelve-months revenue. Achieving profitability on approximately \$35 million of invested equity is a very favorable investment profile. Targeting a value recognition event via a potential acquisition could provide a very significant return to Series B investors. In addition, what differentiates us is a qualified syndicate of Series A investors. Sanderling Ventures is one of the world's oldest and most experienced venture capital firms and Lineagen's board member from Sander-

ling is Fred Middleton, who was the first CFO of Genentech and took Genentech public back in 1980. Another board member is Dinesh Patel from Salt Lake City's Signal Peak Ventures, previously vSpring Capital, who built a company called Theratech and sold that to Watson Pharmaceuticals for over \$300 million. In addition to a great Board of Directors, we have de-risked the deal and this is important for investors. We de-risk the scientific aspects of the deal through our relationships with the Children's Hospital of Philadelphia, University of Utah, and through the previous investment in successfully completed research. We de-risk the medical aspects because the type of test Lineagen uses is recommended by medical organizations. It is de-risked on

the revenue side by positive coverage policies being issued by insurance companies for the type of technology that Lineagen utilizes. While there are non "no-risk" deals in the marketplace, we believe Lineagen provides a unique, risk-mitigated investment profile, a great syndicate of investors, a great management team, and an IP barrier to entry to the most comprehensive and most clinically informative genetic test on the market for individuals with autism and other disorders of childhood development.



**Lineagen, Inc.  
423 Wakara Way, Suite 200.  
Salt Lake City, Utah 84108  
801-931-6200.  
[www.lineagen.com](http://www.lineagen.com)**