



SkylineDx Presented New Algorithm Demonstrating Ability to Successfully Identify Gene Sets that are Informative of Cancer Treatment Specific Survival

Presentation at BioSB 2017 Demonstrated Value of TOPSPIN for Identifying Patient Subgroups Likely to Benefit from Anti-Cancer Treatment

Rotterdam, the Netherlands and Laguna Hills, CA, April 10, 2017 – SkylineDx announced that they had developed TOPSPIN (Treatment Outcome Prediction using Similarity between PatieNts) algorithm for identifying subgroups of patients with multiple myeloma (MM) who are likely to benefit from a new anti-cancer treatment. In the presentation at the BioSB 2017 conference in Lunteren, the Netherlands, researchers described how they used SkylineDx's MMprofiler™ technology and data to develop TOPSPIN, a new computational algorithm to identify gene sets that predict if a patient is likely to survive longer when receiving a treatment of interest versus alternative treatments.

“The wide variance in response rates to multiple myeloma therapies makes it critical to select the right treatment at the time of diagnosis. This requires discovery of predictive biomarkers, such as a gene expression signature, that can predict survival based on which treatment patients receive,” said Joske Ubels, a PhD candidate at SkylineDx, Erasmus Medical Center and the University Medical Center Utrecht. “Finding predictive biomarkers is a particularly difficult machine learning challenge, because currently it is unknown which patients have a survival benefit from a treatment, and which patients have no survival benefit. Moreover, the large amounts of gene expression data generated using platforms such as the MMprofiler poses a computational challenge. To overcome this, we developed TOPSPIN, a new smart search strategy, which leverages biological knowledge combined with high performance computing. We can use the TOPSPIN algorithm to identify gene sets that inform treatment-specific survival, enabling categorization of patients into two subgroups, one of which experiences significantly greater benefit from the treatment of interest than the other class.”

In her BioSB presentation, Ms. Ubels demonstrated the utility of the TOPSPIN method in a MM dataset, in which 910 patients either received (n = 396) or did not receive bortezomib therapy (n = 514). She and her colleagues defined gene sets using the Gene Ontology (GO) annotation, which provides functionally related groups of genes. They then tested random gene sets with the same structure as the GO sets. TOPSPIN successfully identified subsets of patients with longer PFS when treated with Bortezomib.

“The new data establish proof of principle for the TOPSPIN algorithm” commented Dharminder S. Chahal, Chief Executive Officer of SkylineDx. “We plan to test TOPSPIN on other datasets to predict response to different treatments in various cancer types. We thus hope to find other gene sets associated with response to specific anticancer agents, knowledge that can help guide therapeutic decision-making.”

About Multiple Myeloma

Multiple myeloma (MM) is a cancer that arises from plasma cells, a type of white blood cell made in the bone marrow. In patients with MM, the plasma cells become abnormal, multiply uncontrollably, and release only one type of antibody – known as M-protein – which has no useful function. It is often through the measurement of M-protein that MM is diagnosed and monitored. Most medical problems related to MM are caused by the build-up of abnormal plasma cells in the bone marrow and the presence of the M-protein in the blood or urine. The most common symptoms of MM include bone pain, recurring infection, kidney damage, and fatigue. According to the World Cancer



Research Fund International, an estimated 114,000 people around the world are diagnosed with MM annually, and the disease represents 0.8% of all cancers globally.

For more information about MM, visit www.hematon.nl/myeloom (*information available in Dutch only*), www.themmr.org, www.myeloma.org.uk, www.mpeurope.org, www.myeloma.org, and www.jsm.gr.jp.

About MMprofiler™

MMprofiler SKY92 prognostic gene signature assesses risk by measuring the activity of 92 MM-related genes that comprise SKY92, the novel, proprietary gene signature. The lead product of SkylineDx, MMprofiler is proven superior to the biomarkers currently used to risk stratify newly diagnosed and relapsed multiple myeloma patients into a “high” or “standard” risk category.¹ Included in a growing number of international treatment guidelines, MMprofiler is CE-IVD registered in Europe and will be coming soon as a laboratory-developed test (LDT) in the United States. For more information, please visit www.mmprofiler.com.

About SkylineDx

SkylineDx is a commercial-stage biotech company based in Rotterdam, the Netherlands. Originally a spin-off of the Erasmus Medical Center in Rotterdam, the company specializes in the development and marketing of innovative gene signature-based prognostic tests to assist healthcare professionals in making personalized treatment decisions for individual patients. These tests are designed to accurately determine the type or status of the disease or to predict a patient’s response to a specific treatment. Based on the test results, healthcare professionals can tailor the treatment to the individual patient. MMprofiler is the company’s lead product. To learn more, please visit www.skylinedx.com.

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¹ Van Beers EH, et al. SKY92 GEP, iFISH, and ISS comparisons for risk stratification in multiple myeloma. Poster p661 presented at 2015 European Hematology Association Congress.