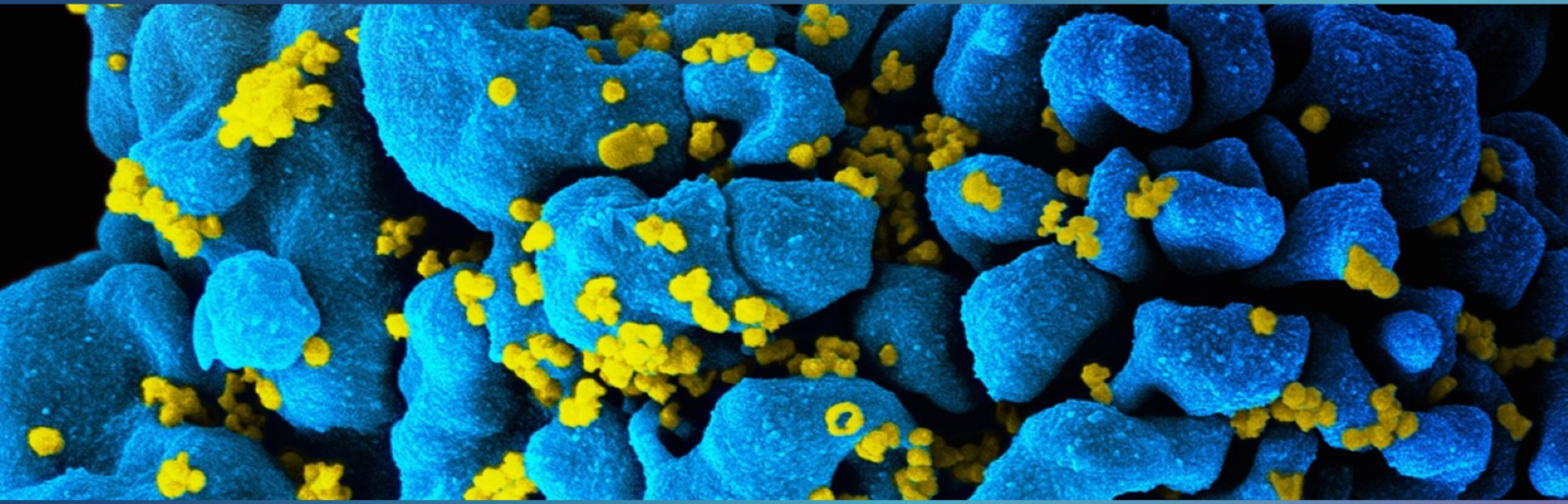
Quanterix®
The Science of Precision Health



Q1 2019 Earnings Call • May 9, 2019

Forward-Looking Statements

Quanterix
The Science of Precision Health

This presentation contains “forward-looking” statements that are based on our beliefs and assumptions and on information available to us as of the date of this presentation. Forward-looking statements include all statements that are not historical facts. We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements. Forward-looking statements involve known and unknown risks, uncertainties, assumptions and other factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements. The risks and uncertainties that we face are described in our most recent filings with the Securities and Exchange Commission. Except as required by law, we assume no obligation to update these forward-looking statements, even if new information becomes available in the future.

Today's Agenda

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The Science of Precision Health

I. Strategic and Financial Progress – Kevin Hrusovsky – Chairman, CEO

   i. Q1 Highlights

   ii. 2019 Goals & Priorities

   iii. Transforming Medicine: Neurology Momentum Update

II. Financial Report – Amol Chaubal – CFO

III. Summary of QTRX Opportunity - Kevin Hrusovsky

IV. Q&A

Massive Market Opportunity, Disruptive Technology and Continued Growth in Revenues & Publications

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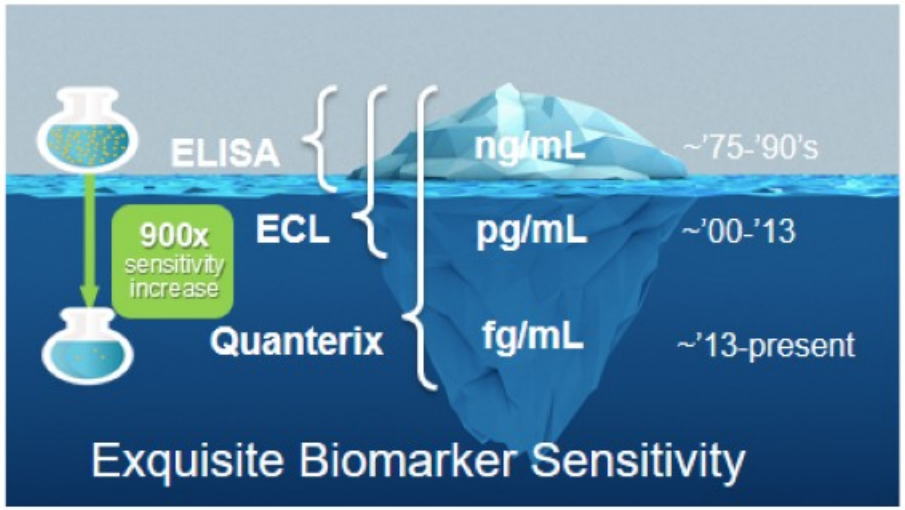
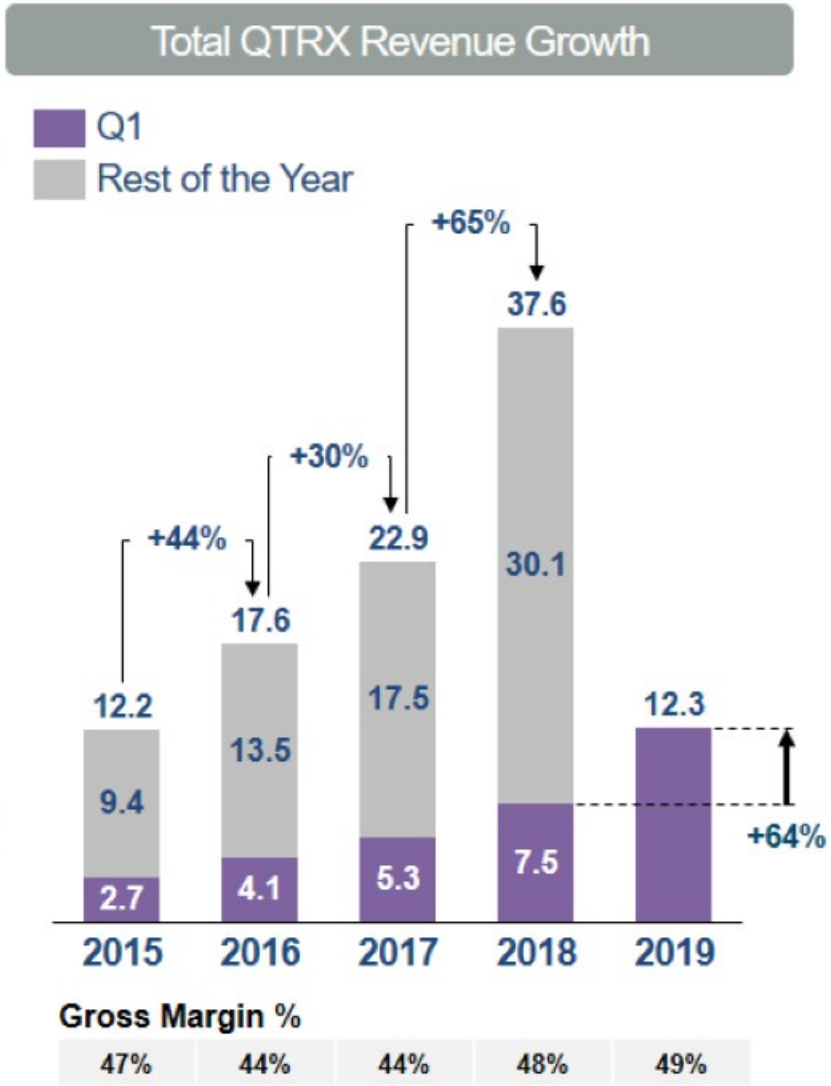


Neuro + Oncology

Research: Today
Market: $1B to $8B
Products to Pharma Services

Diagnostics: 2020+
Market: $5B to $40B
LDT → IVD → DTC



Q1 Highlights

- 64% Revenue growth YOY
- 49% Gross Margin vs 42% PY
- Hired Amol Chaubal, CFO, Mary Ellen Cortizas, Accelerator/CLIA Lab
- Launched SP-X, CorPlex 10 Plex, 9 m-plex assays, 52 onco / immune markers
- 61 new Simoa peer reviewed publications, bringing total >500
  - sNf-L elevation detected in Alzheimer's 16 years before symptoms
  - LIF biomarker offers promise for blood test for pancreatic cancer

Q1 2019 Growth Led by Consumables and Instruments

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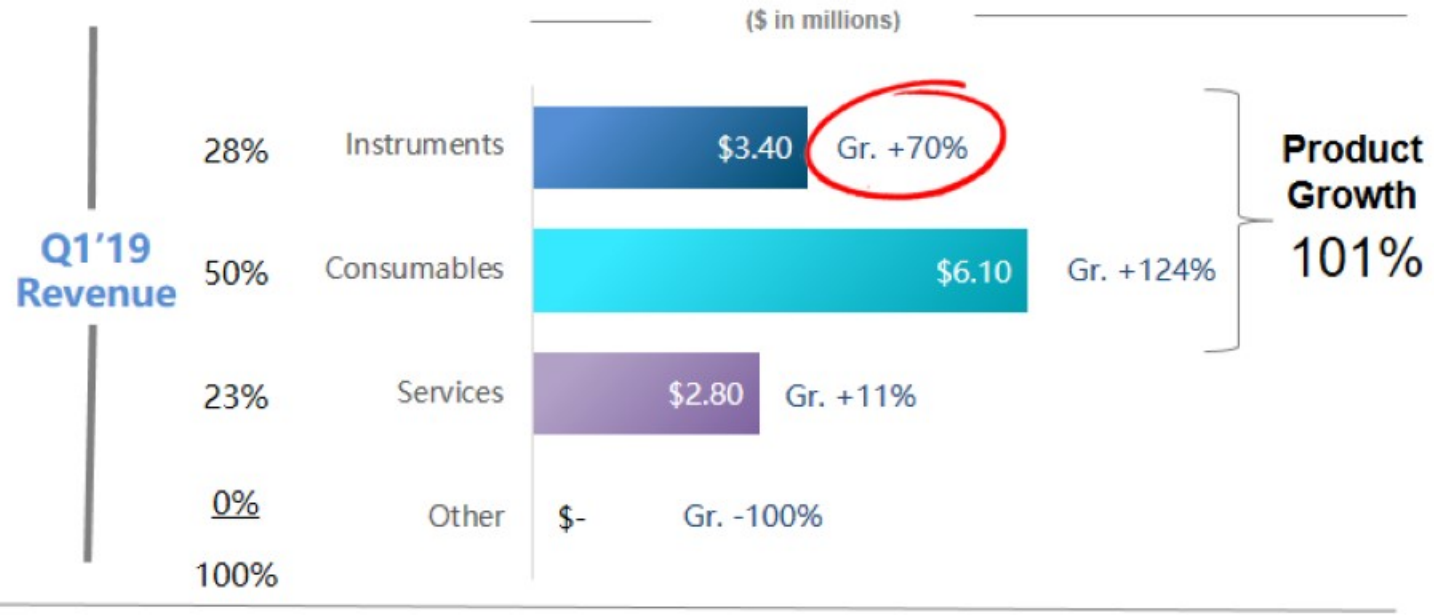
- **64% Growth**
- **High margin mix accelerating**
- Consumables growth +124%; GM 48.2%, +450bp
- Total GM 48.7%, +650bp



Scientific Research is Driving Brand Awareness, Performance and Utilization

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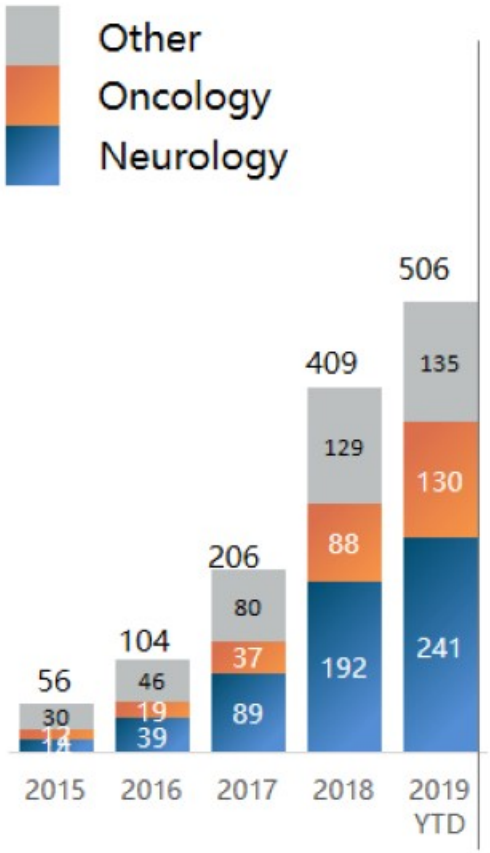
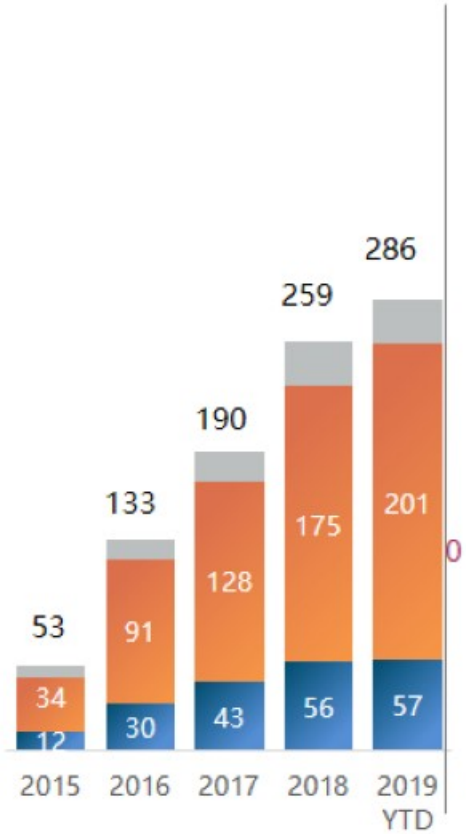
PUBLICATIONS**



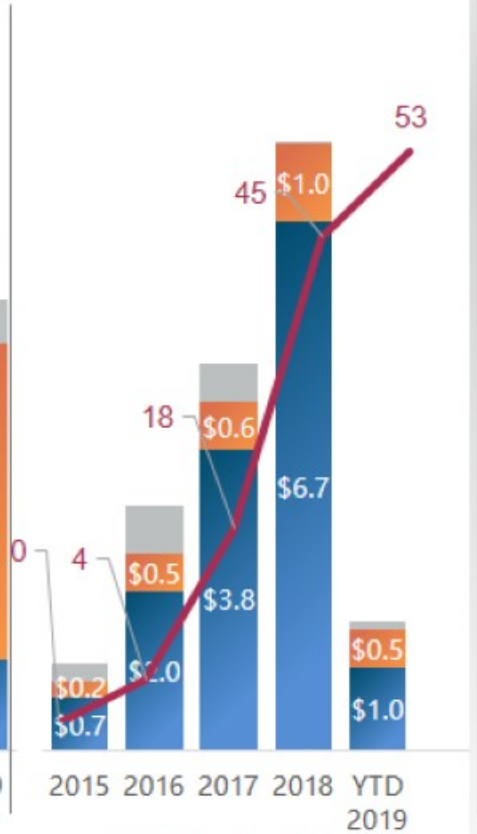
**Cumulative

MARKERS**



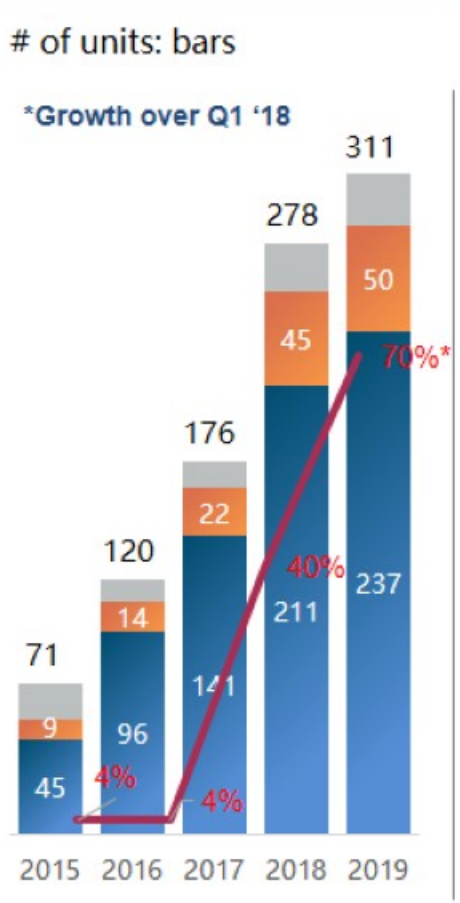
**Cumulative

ACCELERATOR



Number of drug trial projects

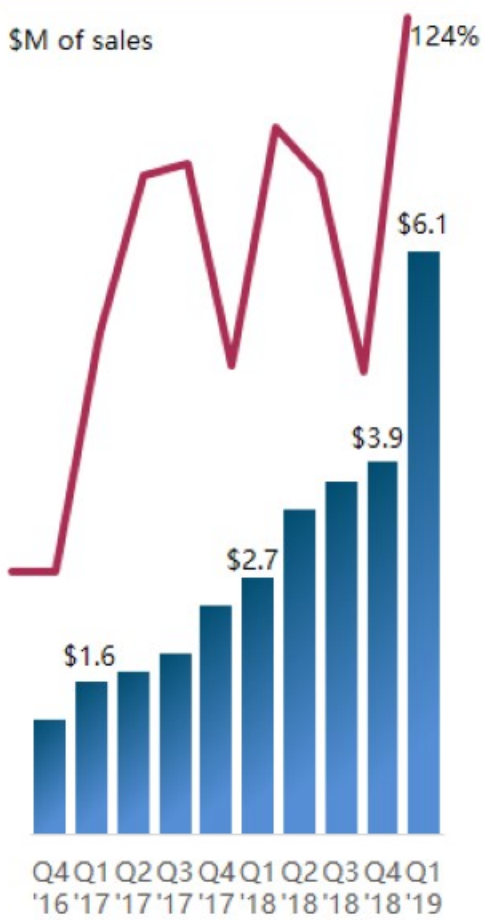
INSTRUMENTS*



Revenue Growth %

*Instrument segmentation estimated based on consumables sold

CONSUMABLES



Consumable growth

Q1 2019 Growth Stratification

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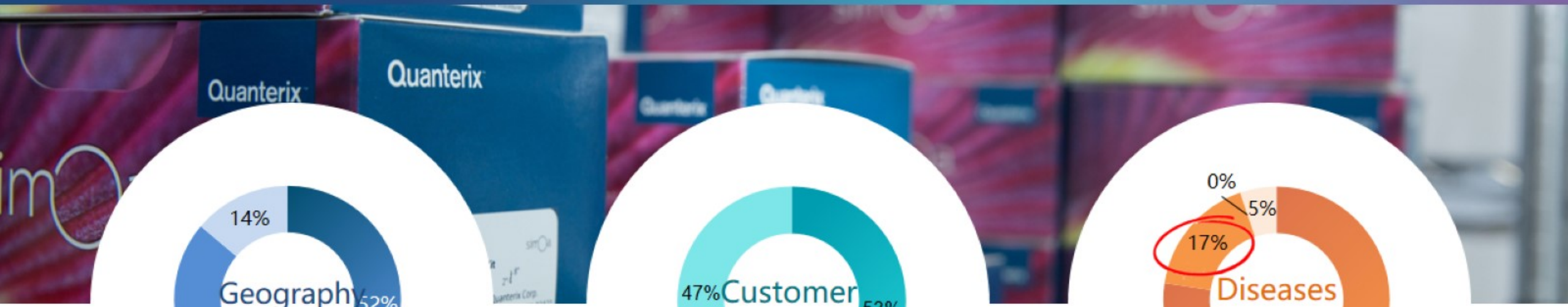

	Growth
NA	+93%
Europe	+96%
Asia	+161%

	Growth
Pharma/Biotech CRO	+126%
Academia	+79%

	Growth	
Neurology	+141%	} 95%
Oncology	+86%	
Cardiology	-82%	
ID	+91%	

2019 Targets & Growth Catalysts On or Ahead of Schedule

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Neurology

High double digit growth

<10% penetrated

Add 25 assays, launch HD-X 4Q'19

Scale globally, LDT/CRO, IT and facilities. Add 50 HC

Oncology

3x Neuro

New entrant

Launch SP-X, CorPlex Assays – 10 plex. 2Q'19

Diagnostics:

50 Drug Trials (neuro/onco)

LDT direct / partner

Explore IVD direct / partner

PPH

Financials:

Growth: 40%

Inst Growth: 25%

Gross Margin: 300 bps

Consumable Utilization 33% of list price or above

Next Generation

- **100x more sensitivity targeted by 2021 YE**
- Lead next frontier of medicine; Protein Translational Modifications

Transforming Medicine with Digital Biomarkers

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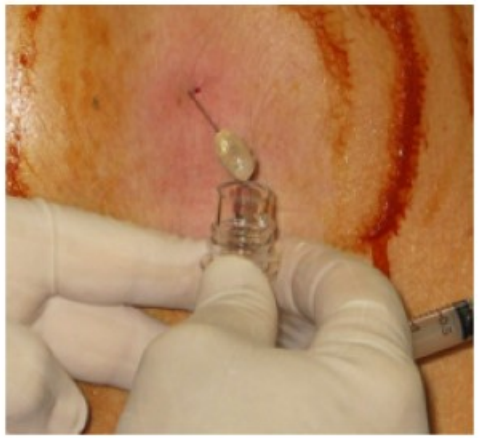
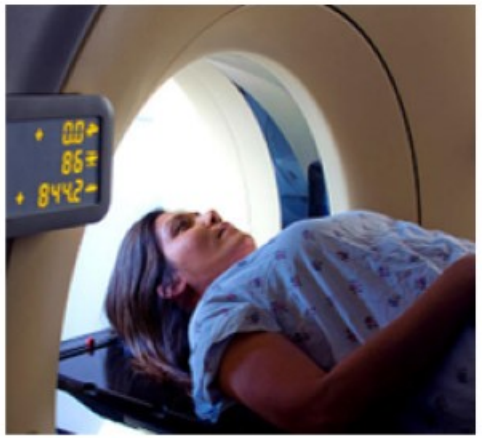
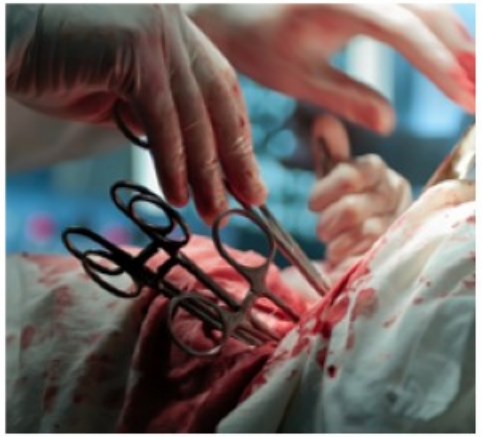
Today: Invasive and Late







Tomorrow: Non-invasive and Early

Simoa Sees Health to Disease Continuum

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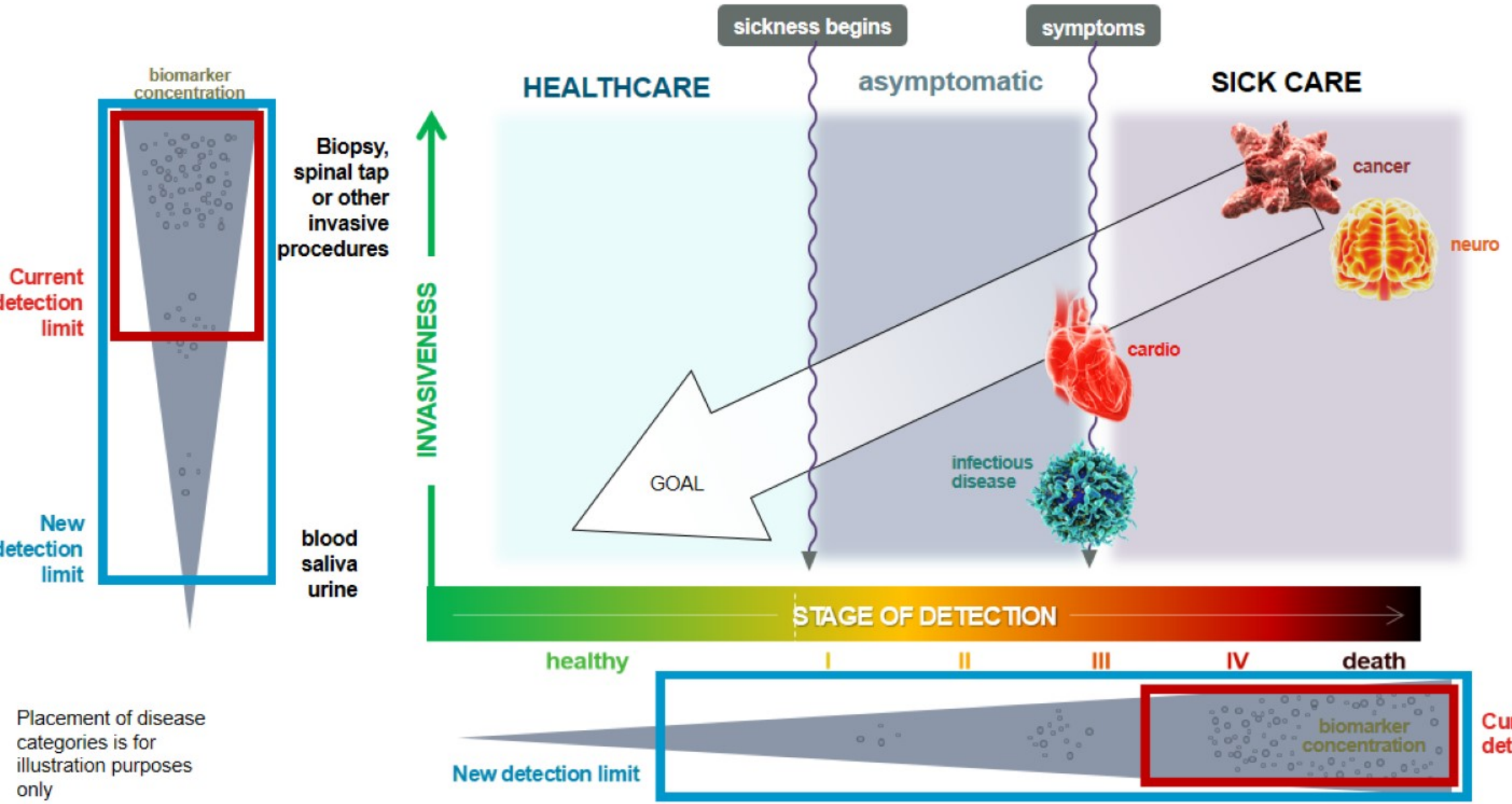

Placement of disease categories is for illustration purposes only

BIOMARKER DISRUPTION - Applications

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	Oncology	Neurology	Cardiology	ID, Inflammation Other*	TOTALS:
	30 pubs 36 markers	170 pubs 50 markers	130 pubs 95 markers		
DEATHS / YR	9MM	18MM	7MM	15MM	**~50MM**
WW COST	$350B	$270B	$470B	$140B	**$1.3T**
BIOMARKER MARKET	$25B	$6B	$9B		**$38B**

Quanterix Product Offerings

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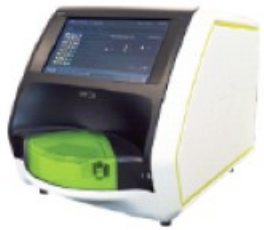
Instruments

HD-1 / HD-X Q4'19

Floor-standing integrated automated system

Assay prep and detection (sample->answer)

400+ publications



SR-X

Benchtop semi-automated assay prep using standardized benchtop devices

SP-X Q2'19

Ultra-sensitive Simoa planar assay technology

Benchtop semi-automated assay prep

Unique multiplex capabilities

Assay kits

Plate based

Bead based

250+ assays for neurology, oncology, cardiology, infectious disease and inflammation

Customization with homebrew kits

Singleplex and multiplex formats

Services

- ✓ Contract research services through Simoa Accelerator Laboratory
- ✓ Sample testing services
- ✓ Custom assay development
- ✓ Custom reagent production and kitting
- ✓ **CLIA and LDT capabilities**

Simoa: Technology and Instrumentation

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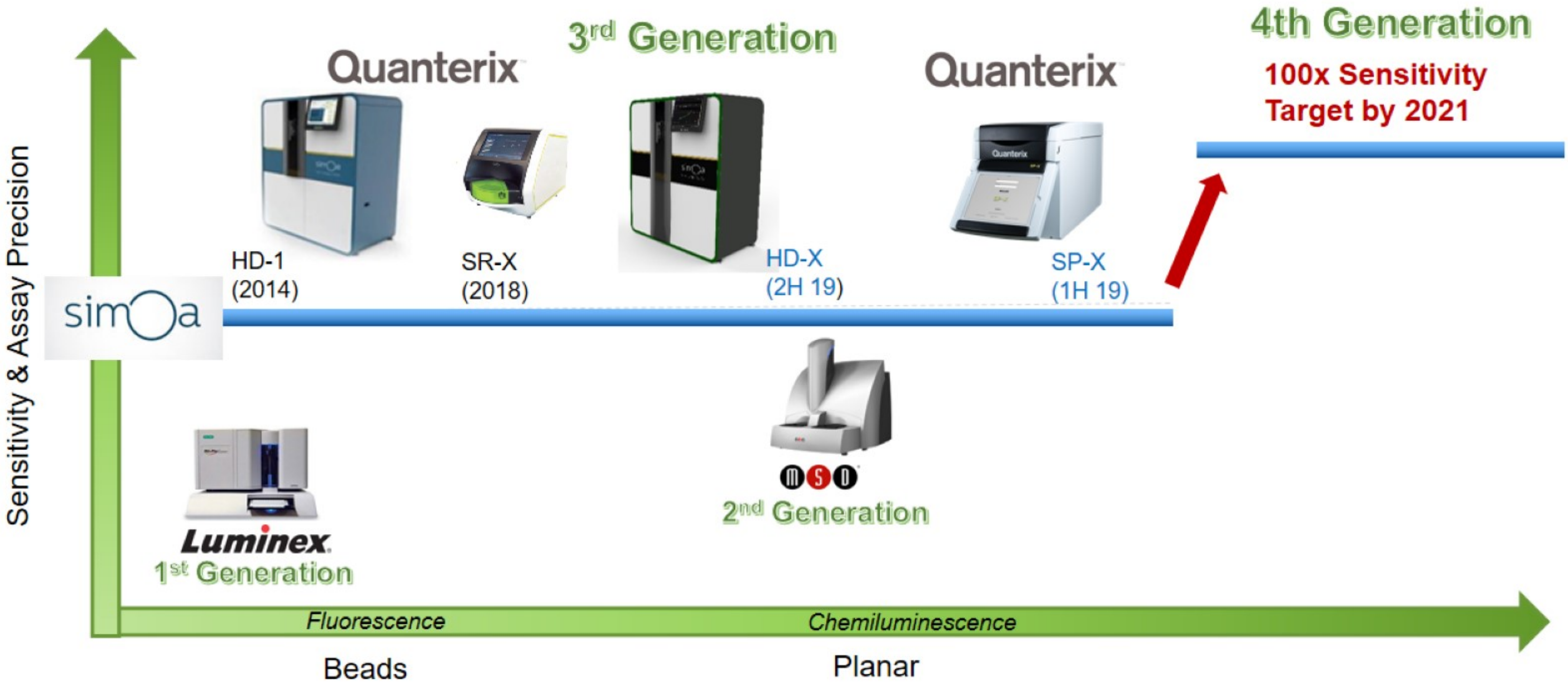

Competitive Landscape

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	Quanterix		Luminex	MilliporeSigma Singulex	MSD	proteinsimple
Technology	Bead-Based Simoa	Planar SP-X Simoa	Bead-based Immunoassay	Erenna Immunoassay	Electrochemi-luminescence	ELLA
Sensitivity	🟢	🟡	🔴	🟢	🔴	🔴
Dynamic Range	🟢	🟢	🟢	🟡	🟢	🟡
Automation & Ease of Use	🟢	🟡	🟡	🔴	🟡	🟢
Precision	🟢	🟢	🔴	🔴	🟡	🟢
Multiplexing	🟡	🟢	🟢	🔴	🟢	🟡
Menu	🟡	🟡	🟢	🔴	🟢	🟡
Cost	🟡	🟢	🟡	🔴	🟡	🟡

We are Addressing a Significant Unmet Need in Drug Development

FDA Announces Office of Drug Evaluation Science - ODES

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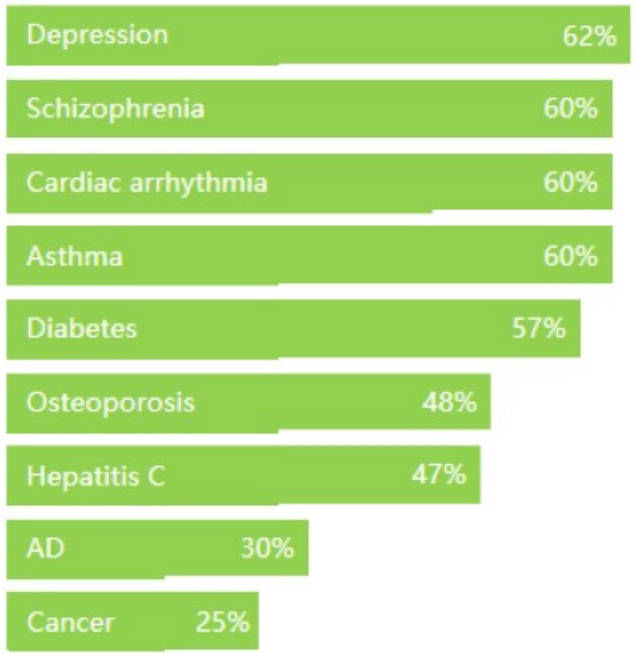
DRUG PERFORMANCE

TOXICITY

Adverse drug events are a substantial cause of Death in USA

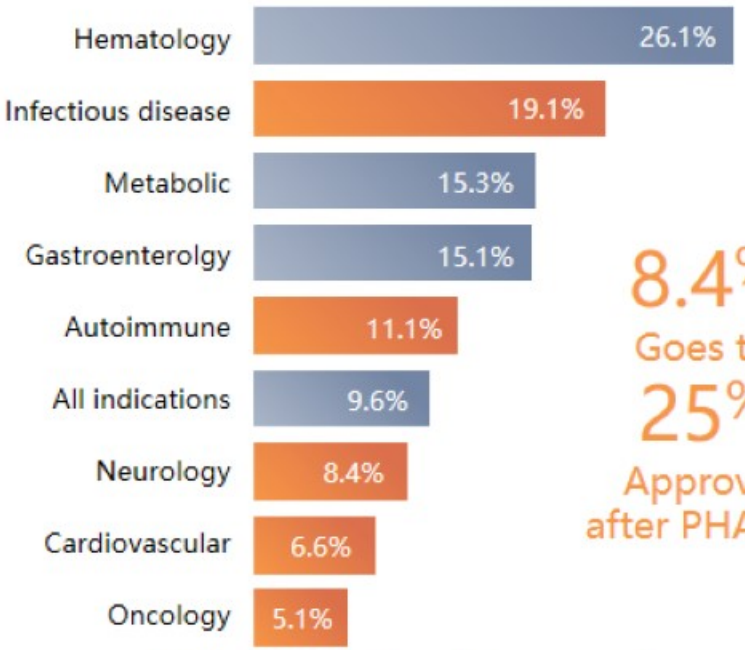
EFFICACY



PROBABILITY OF DRUG APPROVAL

300%

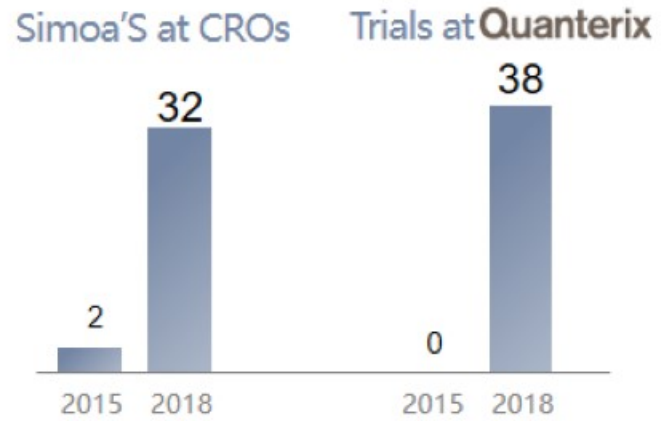
increase if biomarkers are used



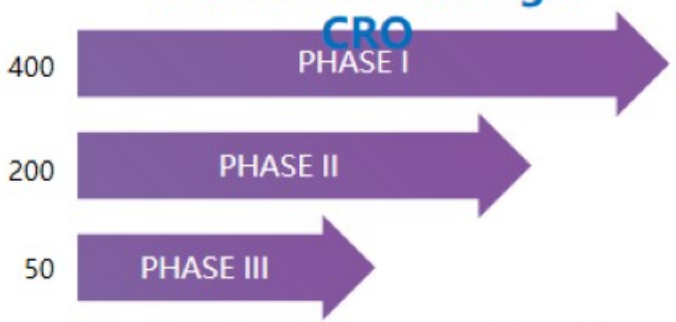
8.4% Goes to **25%** Approval after PHASE I

Probability of phase III approval after Phase 1 approval

VALIDATION OF SIMOA IMPACT



650 clinical trials with Simoa at single CRO



MYRIAD RBM

Business-Favorable Changes in IVD Regulatory Climate

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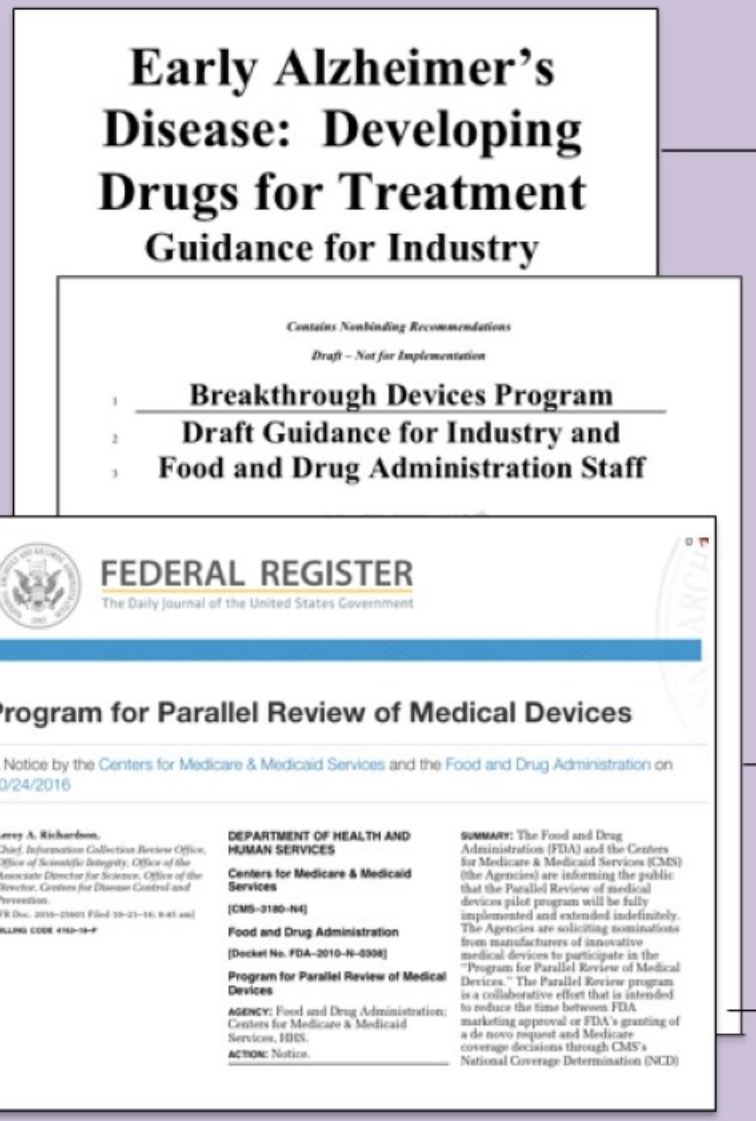

FDA INITIATIVE...	WHAT IT MEANS FOR QTRX...
Recognizes the criticality of using biomarkers.	**Encourages biopharma to identify/incorporate promising biomarkers in trial design**
GUIDANCE DOCUMENT **Alzheimer's Disease: Developing Drugs for Treatment** **Guidance for Industry FEBRUARY 2018** **ENDPOINTS FOR STAGE 3,2,1 TRIALS** **CDER: BILLY DUNN**	
Expedited development, assessment, and review	**Expedited review of IVD tests; more probable Class II path. Example: Banyan TBI test (6 mo to clearance).**
FDA teaming up with CMS to provide parallel review of medical device approval and Medicare coverage.	**Potential to secure reimbursement determination simultaneous with FDA clearance**

Blue Chip Customers

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Research Institutions

Washington University in St. Louis

Penn

University of Gothenburg

Vejle Hospital
part of Lillebaelt Hospital

Cedars-Sinai

Institut Pasteur

Wyss Institute

Biopharma

Lilly

Biogen

MERCK

axon NEUROSCIENCE

Boehringer Ingelheim

AMGEN

Other

MYRIAD RBM

BIOMÉRIEUX

Blue Chip Customers

Quanterix
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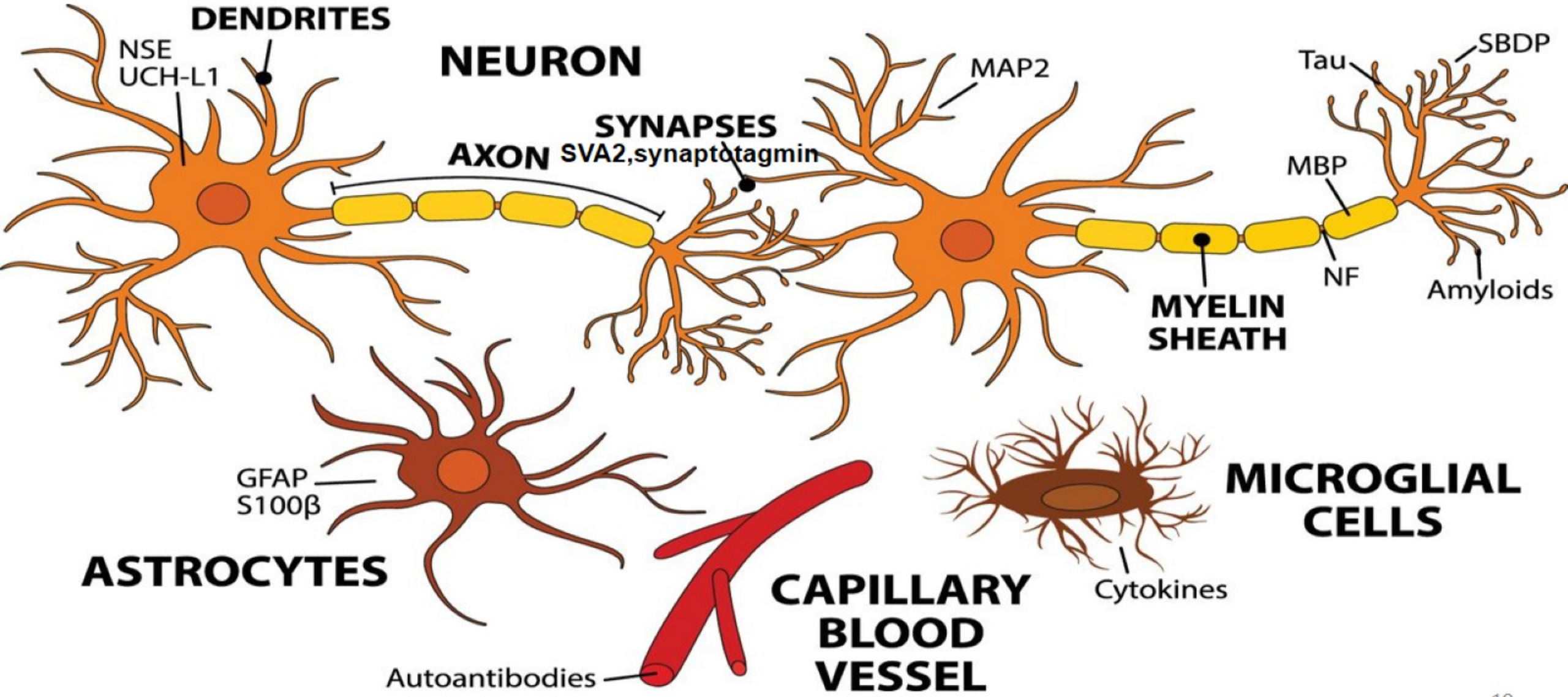
Research Institutions

Biopharma

Other

2018


DENDRITES
NSE
UCH-L1
NEURON
MAP2
SYNAPSES
AXON
SVA2,synaptotagmin
Tau
SBDP
MBP
NF
Amyloids
MYELIN
SHEATH
GFAP
S100β
ASTROCYTES
MICROGLIAL
CELLS
Cytokines
CAPILLARY
BLOOD
VESSEL
Autoantibodies

Neurology Momentum Fueling Growth and Future Strategy

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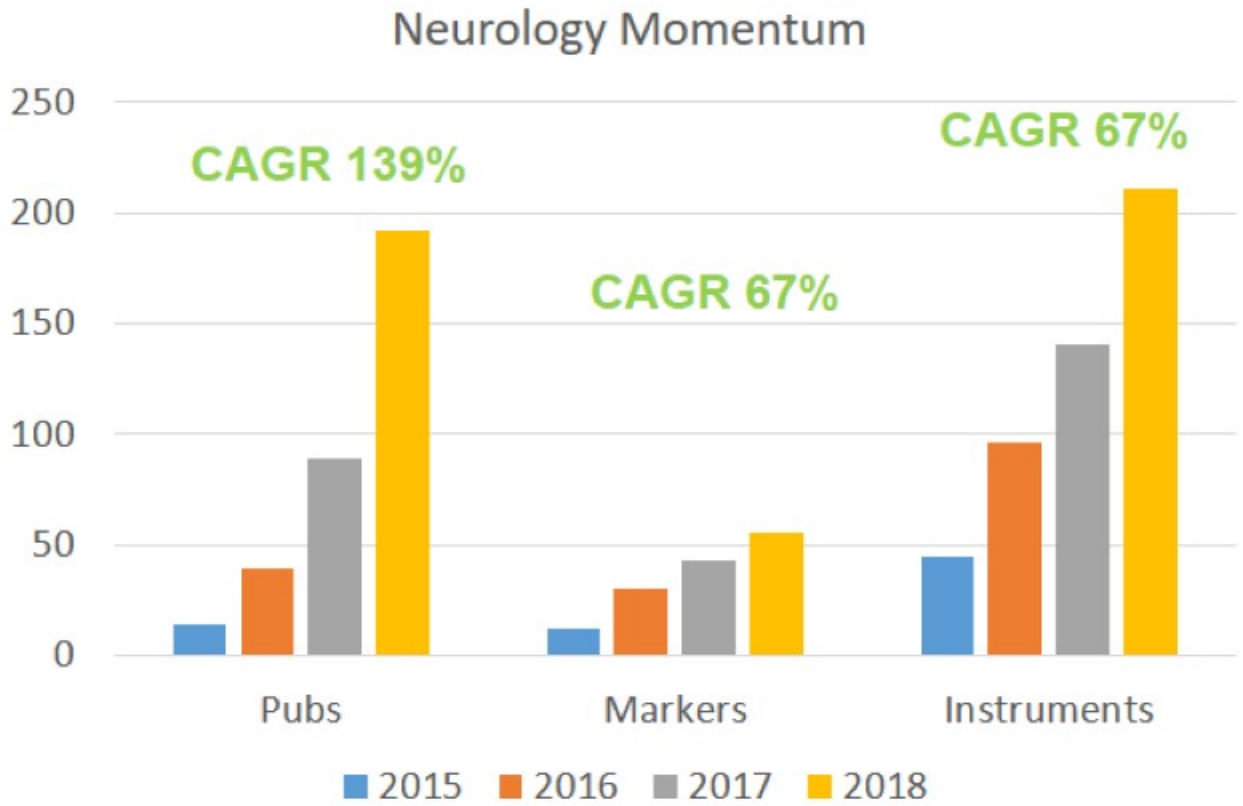

- Strongest **growth in neurology** RUO franchise with **accelerating publications** 2018
- **Nf-L, amyloid beta, tau**, and other markers showing tremendous **potential for key clinical applications** in top peer-reviewed pubs
- Strong network of **world-leading KOLs**
- Inbound **FDA interest** (Nf-L); recent **breakthrough device exemption** for AB40/42 mass spec test for Alzheimer's
- **Media, patient advocacy, and public attention** to promise of blood-based neurology tests (CNN, Forbes, Bloomberg, Washington Post, GMA, etc.
- **Deep adoption** by leading academic and pharma

GOOD MORNING AMERICA · Lilly · UBC · Bloomberg · HEAD HEALTH CHALLENGE · NFL · Biogen · SANOFI · Genentech

NF-L Rapidly Expanding as Best In Class Neuro Biomarker

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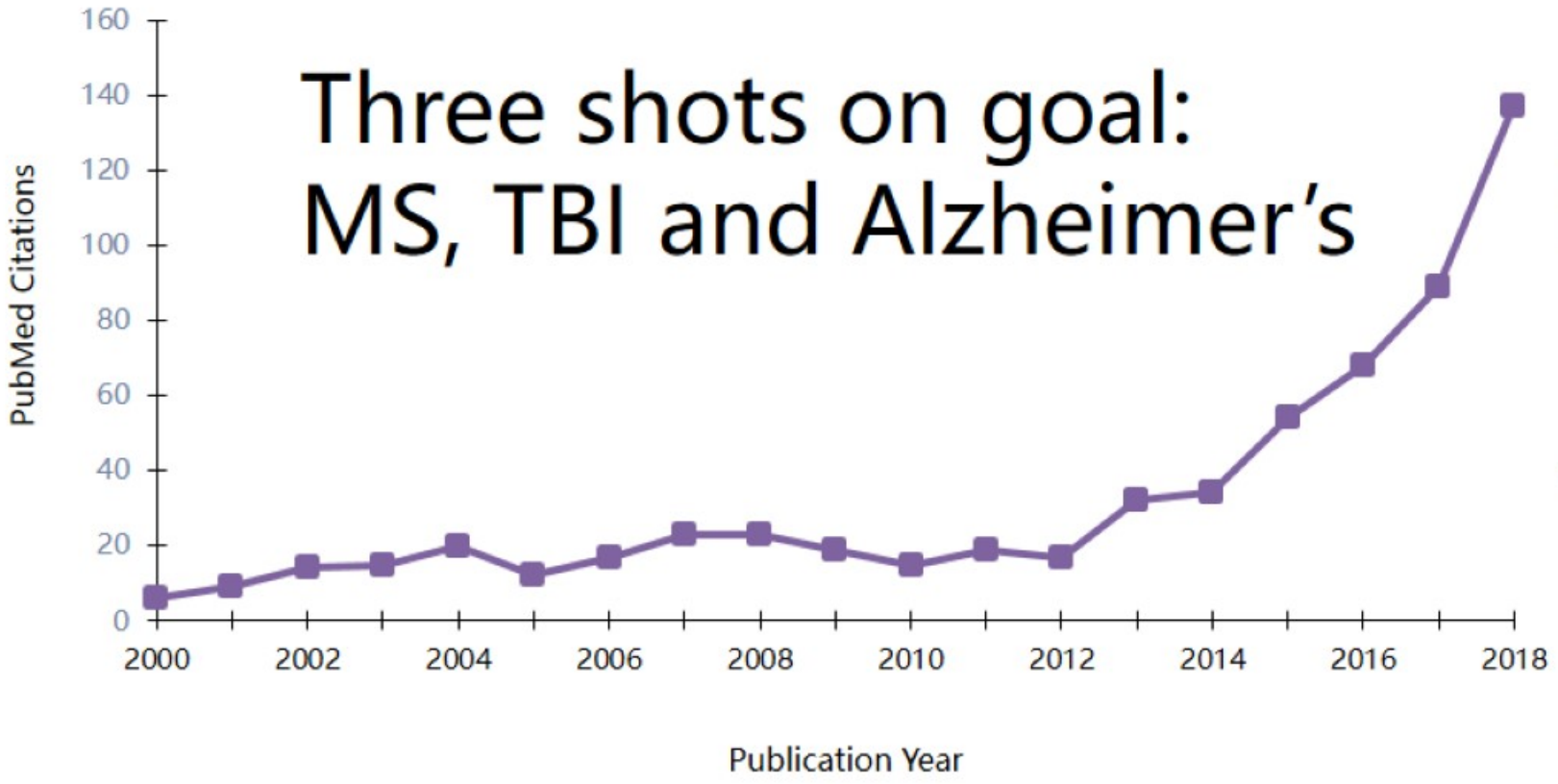
RESEARCH PUBLICATIONS ON NFL



Three shots on goal:
MS, TBI and Alzheimer's

46 active clinical trials using sNf-L

Genentech
A Member of the Roche Group
Lilly
Pfizer
Biogen
NOVARTIS
SANOFI

Source: Health Advances analysis, PubMed.

Emerging Clinical Biomarker: Neurofilament Light (NfL)

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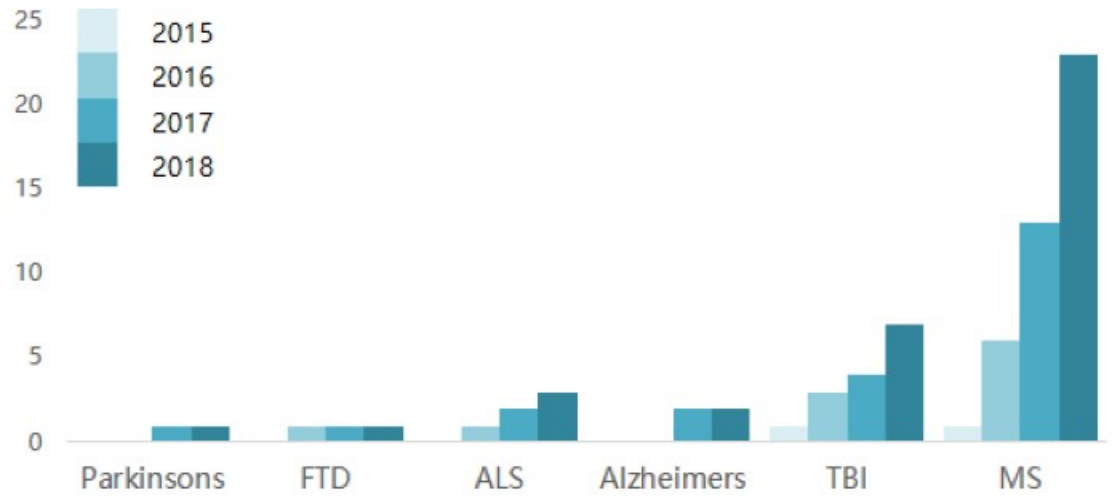
NFL PUBLICATIONS

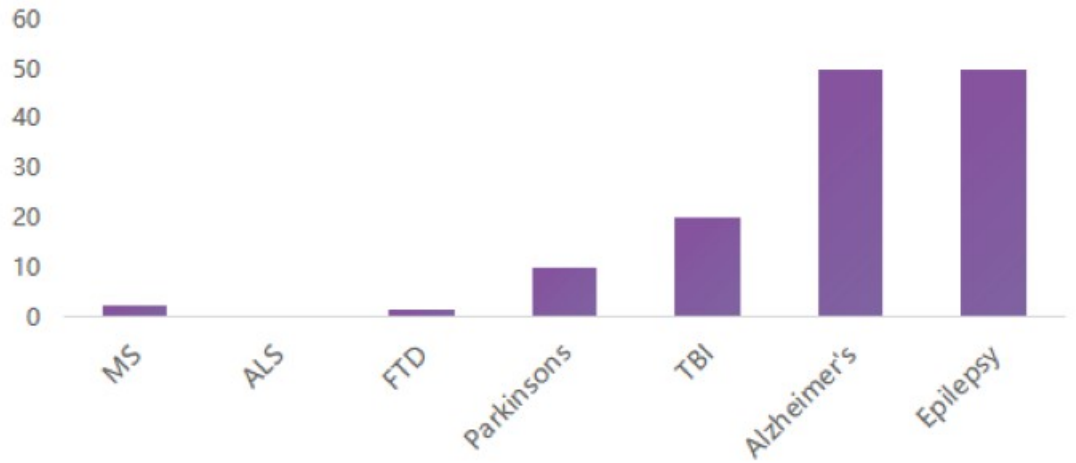


STUDIES CONFIRM NFL CLINICAL UTILITY:

- Disease activity monitoring
- Drug efficacy monitoring
- Relapse/severity prognostic

Majority of published data obtained with Simoa NfL

WW DISEASE INCIDENCE (MILLIONS)



MULTIPLE SCLEROSIS:

- Avg. age of onset: 34 yrs; avg. life expectancy after onset: 30 yrs
- Standard of care: MRI 1-2X/yr
- NfL as MRI replacement: 3.5M tests/yr

Clinical Vaildation of NfL for MS is a Key Beachhead

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Neurology®

Blood neurofilament light chain as a biomarker of MS disease activity and treatment response

Jens Kuhle, MD,* Harald Kropshofer, PhD,* Dieter A. Haering, PhD, Uma Kundu, MPharm, Rolf Meinert, PhD, Christian Barro, MD, Frank Dahlke, MD, Davorka Tomic, PhD, David Leppert, MD, and Ludwig Kappos, MD

Published February 8, 2019

Conclusions:

Blood NfL levels are associated with clinical and MRI-related measures of disease activity and neuroaxonal damage and have prognostic value. Our results support the utility of blood NfL as an easily accessible biomarker of disease evolution and treatment response.

JAMA Neurology

Association Between Longitudinal Plasma Neurofilament Light and Neurodegeneration in Patients With Alzheimer's Disease

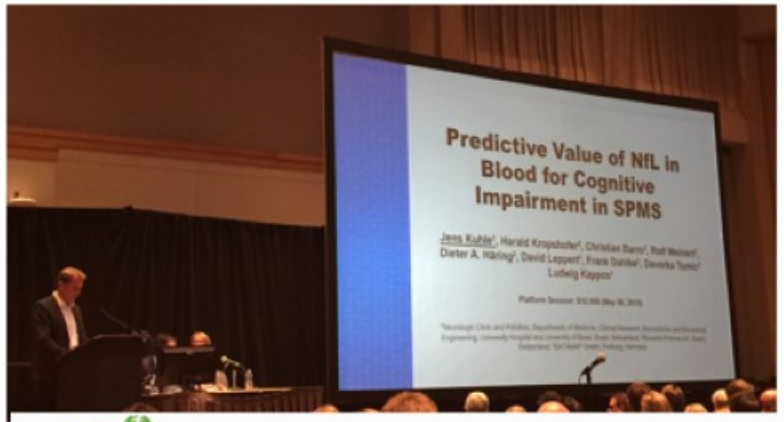
Niklas Mattsson, MD, PhD; Nicholas C. Cullen, BSc; Ulf Andreasson, PhD; Henrik Zetterberg, MD, PhD; Kaj Blennow, MD, PhD

JAMA Neurol. doi:10.1001/jamaneurol.2019.0765

Conclusions:

The findings suggest that plasma NfL can be used as a noninvasive biomarker associated with neurodegeneration in patients with AD and may be useful to monitor effects in trials of disease-modifying drugs.

AAN 2019: Explosive momentum of Simoa sNf-L



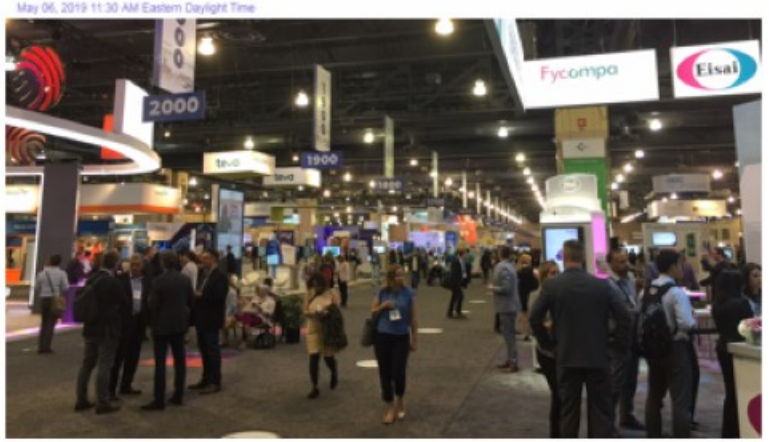
BusinessWire
A Berkshire Hathaway Company

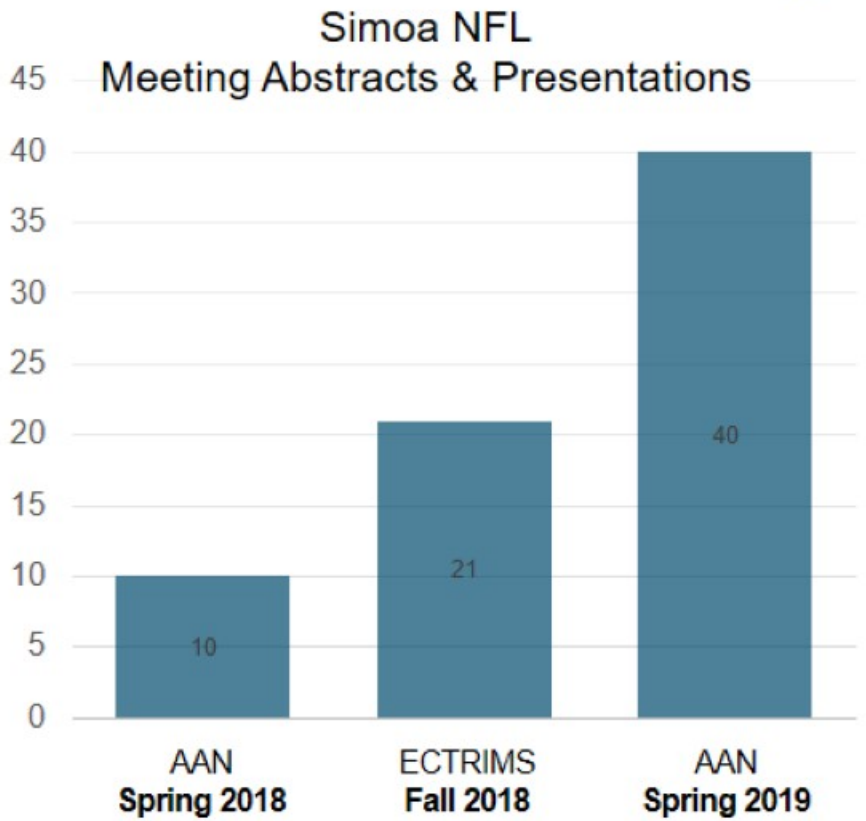
HOME SERVICES NEWS EDUCATION ABOUT US Search

Quanterix' Simoa Technology Powers More Than 85 Percent of Neurofilament Light Biomarker Research to be Unveiled at American Academy of Neurology Annual Meeting

Leading global neurology conference will feature 36 new Simoa-powered studies validating the use of Neurofilament light chain (Nf-L) as a potential diagnostic and prognostic biomarker for neurodegeneration

May 06, 2019 11:30 AM Eastern Daylight Time





5 Phase III trials across >1600 MS patients demonstrate clinical utility of Simoa NFL for relapsing remitting MS disease monitoring

Serum Neurofilament Light (NfL) for Disease Prognosis and Treatment Monitoring in Multiple Sclerosis Patients: Toward Implementation Into Clinical Care.

Peter A. Calabresi,[1] Jens Kuhle,[2] Douglas L. Arnold,[3] R. Philip Kinkel,[4] Ludwig Kappos,[2] Carol M. Singh,[5] Dipen Sangurdekar,[6] Carl De Moor,[7] Bob Engle,[8] Ray Su,[8] Aaron Deykin,[5] Elizabeth Fisher,[5] Alfred Sandrock,[5] Bernd C. Kieseier,[9] Richard A. Rudick,[5] Tatiana Plavina[5]

[1]Department of Neurology, Johns Hopkins University School of Medicine, Baltimore, MD, USA; [2]Neurologic Clinic and Policlinic, Departments of Medicine, Biomedicine and Clinical Research, University Hospital Basel, Basel, Switzerland; [3]Montreal Neurological Institute, McGill University, Montreal, QC, Canada; [4]Department of Neurosciences, University of California, San Diego, CA, USA; [5]Biogen Inc., Cambridge, MA, USA

Natalizumab Reduces Serum Concentrations of Neurofilament Light Chain in Secondary Progressive Multiple Sclerosis Patients From the Phase 3 ASCEND Study

Raju Kapoor[1], Finn Sellebjerg[2], Hans-Peter Hartung[3], Douglas Arnold[4], Mark S. Freedman[5], Douglas Jeffery[6], Aaron Miller[7], Keith R. Edwards[8], Carol M. Singh[9], In Chang[10], Zhang Ren[11], Dipen Sangurdekar[11], Bing Zhu[11], Devangi Mehta[11], Pei-Ran Ho[11], Nolan Campbell[11], Michael Edwards[11], Elizabeth Fisher[11], Bernd C. Kieseier[12], Richard A. Rudick[11], Tatiana Plavina[11]

Long-term Effect of Fingolimod in Reducing Blood Neurofilament Light Levels in Patients with Relapsing-remitting Multiple Sclerosis

Jeffrey Cohen[1], Ludwig Kappos[2], Nadia Tenenbaum[3], Jackie Han[3], Harald Kropshofer[4], Davorka Tomic[4], Jens Kuhle[2]
[1]Cleveland Clinic, [2]Neurologic Clinic and Policlinic, Department of Medicine, Clinical Research, Biomedicine and Biomedical Engineering, University Hospital and University of Basel, [3]Novartis Pharmaceuticals Corporation, [4]Novartis Pharma AG

Objective:
To assess the effect of long-term treatment with fingolimod on blood neurofilament light chain (NfL) levels in patients with relapsing-remitting multiple sclerosis (RRMS).
Background:
NfL, a cytoskeleton protein, is elevated in blood upon neuroaxonal damage. Blood NfL is a promising biomarker for monitoring disease activity, treatment response, and prognosis in MS.
Design/Methods:
This post hoc analysis was based on data from patients who received fingolimod 0.5 mg once daily or placebo/interferon ß-1a (IFN) 30 µg once weekly in pivotal studies (24-month FREEDOMS/12-month TRANSFORMS), and then fingolimod in the open-label LONGTERMS extension study for up to 10 years. The analysis included a subset of patients who had blood NfL assessments at baseline, end of core (EoC) in pivotal studies, and end of study (EoS) in LONGTERMS. Patients were categorized into two groups: a continuous group (n=37) who received fingolimod throughout the studies and a switched group (n=42) who transitioned from placebo/IFN group to fingolimod in the LONGTERMS. NfL was measured using Single Molecule Array (Simoa™) immunoassay. The geometric mean change in NfL levels from baseline to EoS was analyzed using Wilcoxon signed-rank test.
Results:
The mean exposure to fingolimod was 3483 days in the continuous group and 2822 days in the switched group. In the continuous group, baseline NfL levels of 33 pg/mL were significantly reduced by approximately 40% at both EoC and EoS (20 pg/mL; P<0.0001 and P<0.0002, respectively). In the switched group, baseline NfL levels of 29 pg/mL were reduced by 15% at EoC (25 pg/mL, P=0.44) and 41% at EoS (17 pg/mL, P<0.0001).
Conclusions: Fingolimod 0.5 mg significantly reduced blood NfL, maintaining its low levels with continuous treatment for up to 10 years. NfL levels were reduced to a lesser extent during treatment with IFN but decreased further with switch to fingolimod, demonstrating the greater impact of highly effective therapy in RRMS.

Simoa NfL Multicenter Analytical Validation - 17 sites across Europe and North America

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	Location	Investigator	Study Site
1	Amsterdam	Dr. Charlotte	Neurochemistry Laboratory, Amsterdam University
2	Barcelona	Dr. Manuel	Laboratori de Neuroinmunologia Clinica Centre
3	Basel	Prof Jens Kuhle, Dr. Zuzanna	Department Biomedicine, Univ Hospital Basel, Switzerland
4	Dresden	Dr. Katja Akgün Dr. Ziemssen, Tjalf	Neuroimmunological lab, Center of Clinical Neuroscience, Dresden, Germany
5	Gothenburg	Prof. Kaj Blennow Prof. Henrik	Clinical Neurochemistry Lab, Mölndal Hospital, Mölndal, Sweden
6	Göttingen	Prof. Wolfgang Dr. Niels Kruse	Institut für Neuropathologie, Universitätsmedizin Göttingen, Germany
7	London	Dr. Lucia Bianchi Prof. Gavin	Dept of Neuroscience & Trauma Blizard Institute Queen Mary Univ of London, UK
8	London	Dr. Amanda Prof. Henrik	The DRI Fluid Biomarker Laboratory at University College London, United Kingdom
9	Mainz	Prof. Stefan Bittner	Klinik für Neurologie, Universitätsmedizin Mainz,
10	Milan	Dr. Comi Giancarlo Prof.Roberto Furlan	Clinical Neuroimmunology Unit - Institute of Experimental Neurology, MIlan, Italy
11	Montpellier	Prof. Markus Otto Dr. Patrick Oeckl	Hôpital St Eloi, Montpellier, France
12	Ulm	Prof. Markus Otto Dr. Patrick Oeckl	University of Ulm, Ulm, Germany
13	Bethesda	Dr. Ruturaj Dr. Bibi Bielekova	National Institutes of Health, Bethesda, MD
14	Ottawa	Dr. Simon Thebault Dr. Freedman, Mark Dr. Booth, Ronald	MS Clinical Ottawa Hospital University of Ottawa, Ottawa ON Canada
15	Philadelphia	Dr. Marcus Handy Dr. Amit Bar-Or	Perelman School of Medicine, University of Pennsylvania, Department of Neurology, Philadelphia,
16	Lexington	Kevin Hrusovsky Dr. David Wilson	Quanterix Corp, Lexington MA
17	Framingham	Dr. Matthew Dr. Martin Kramer	Sanofi Genzyme, Framingham MA

NFL Normative and MS Study - Swiss Multiple Sclerosis Registry

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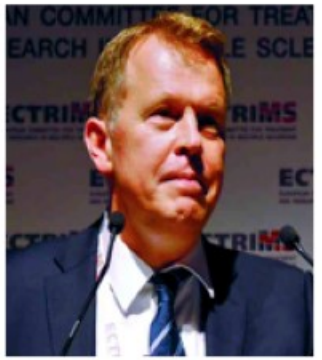
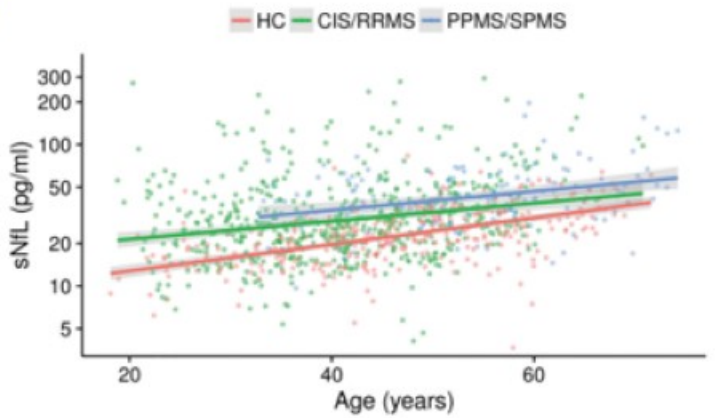
Universitätsspital Basel



- Prof. Jens Kuhle, MD, PhD, and Prof. David Leppert, PhD
- 10 collaborating institutions
- 18,000 samples across demographics (2019)
- 7,000-8,000 MS samples (2020-2021)
- Key objective: define what is 'normal' NFL for different ethnicities and age groups
- Partially supported by Quanterix
- Will solidify Simoa NFL as the go-to test for screening and monitoring brain health



RESEARCH ARTICLE

Serum Neurofilament Light: A Biomarker of Neuronal Damage in Multiple Sclerosis

Giulio Disanto, MD, PhD,[1] Christian Barro, MD,[2] Pascal Benkert, PhD,[3] Yvonne Naegelin, MD,[2] Sabine Schädelin, MSc,[3] Antonella Giardiello, MD,[1] Chiara Zecca, MD,[1] Kaj Blennow, PhD,[4] Henrik Zetterberg, PhD,[4,5] David Leppert, MD,[2] Ludwig Kappos, MD,[2] Claudio Gobbi, MD,[1] Jens Kuhle, MD, PhD,[2] and the Swiss Multiple Sclerosis Cohort Study Group

Objective: Neurofilament light chains (NfL) are unique to neuronal cells, are shed to the cerebrospinal fluid (CSF), and are detectable at low concentrations in peripheral blood. Various diseases causing neuronal damage have resulted in elevated CSF concentrations. We explored the value of an ultrasensitive single-molecule array (Simoa) serum NfL (sNfL) assay in multiple sclerosis (MS).

Methods: sNfL levels were measured in healthy controls (HC, $n = 254$) and two independent MS cohorts: (1) cross-sectional with paired serum and CSF samples ($n = 142$), and (2) longitudinal with repeated serum sampling ($n = 246$, median follow-up = 3.1 years, interquartile range [IQR] = 2.0–4.0). We assessed their relation to concurrent clinical, imaging, and treatment parameters and to future clinical outcomes.

Results: sNfL levels were higher in both MS cohorts than in HC ($p < 0.001$). We found a strong association between CSF NfL and sNfL ($\beta = 0.589$, $p < 0.001$). Patients with either brain or spinal (43.4pg/ml, IQR = 25.2–65.3) or both brain and spinal gadolinium-enhancing lesions (62.5pg/ml, IQR = 42.7–71.4) had higher sNfL than those without (29.6pg/ml, IQR = 20.9–41.8; $\beta = 1.461$, $p = 0.005$ and $\beta = 1.902$, $p = 0.002$, respectively). sNfL was independently associated with Expanded Disability Status Scale (EDSS) assessments ($\beta = 1.105$, $p < 0.001$) and presence of relapses ($\beta = 1.430$, $p < 0.001$). sNfL levels were lower under disease-modifying treatment ($\beta = 0.818$, $p = 0.003$). Patients with sNfL levels above the 80th, 90th, 95th, 97.5th, and 99th HC-based percentiles had higher risk of relapses (97.5th percentile: incidence rate ratio = 1.94, 95% confidence interval [CI] = 1.21–3.10, $p = 0.006$) and EDSS worsening (97.5th percentile: OR = 2.41, 95% CI = 1.07–5.42, $p = 0.034$).

Interpretation: These results support the value of sNfL as a sensitive and clinically meaningful blood biomarker to monitor tissue damage and the effects of therapies in MS.

ANN NEUROL 2017;81:857–870

"There is an urgent unmet need for reliable biomarkers of neurodegeneration. NFL addresses this need."

- Prof Jens Kuhle

Desanto et. al. , Ann Neurol. 2017 Jun; 81(6): 857–870.

Blood Test May Detect Alzheimer's In Patients 16 Years Before Symptoms Appear

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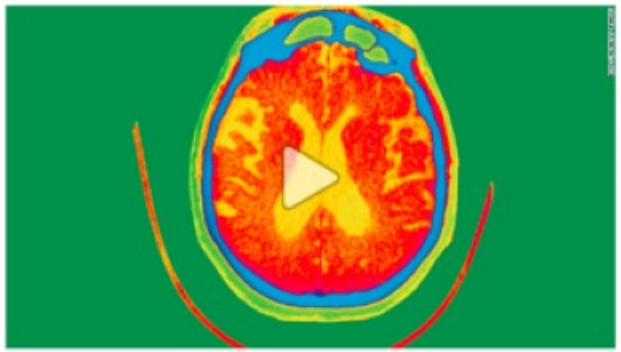
CNN Health | Food | Fitness | Wellness | Parenting | Live Longer — Live TV | U.S. Edition

Blood test could detect Alzheimer's up to 16 years before symptoms begin, study says

By Nina Avramova, CNN
Updated 2:46 PM ET, Tue January 22, 2019



How to prevent Alzheimer's disease 03:33

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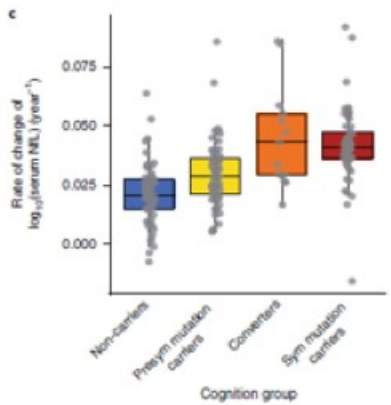
(CNN) — A simple blood test could predict if a patient will develop Alzheimer's disease up to 16 years before symptoms begin, a new study finds.

By measuring changes in the levels of a protein in the blood, called neurofilament light chain (NfL), researchers believe any rise in levels of the protein could be an early sign of the disease, according to the study published Monday in the journal Nature Medicine.

NfL is a "marker in the blood which gives an indication of nerve cell loss in the brain," explained lead researcher Mathias Jucker, professor of cell biology of neurological diseases at the German Center for Neurodegenerative Diseases. "The more neurofilament you have in the blood, the more brain damage you have," he said.



nature medicine — LETTERS
https://doi.org/10.1038/s41591-018-0304-3

Serum neurofilament dynamics predicts neurodegeneration and clinical progression in presymptomatic Alzheimer's disease

Oliver Preische[1,2,21], Stephanie A. Schultz[3,21], Anja Apel[1,2,21], Jens Kuhle[4], Stephan A. Kaeser[1,2], Christian Barro[4], Susanne Gräber[1], Elke Kuder-Buletta[1], Christian LaFougere[1], Christoph Laske[1,2], Jonathan Vöglein[5,6], Johannes Levin[5,6], Colin L. Masters[7], Ralph Martins[8,9], Peter R. Schofield[10,11], Martin N. Rossor[12], Neill R. Graff-Radford[13], Stephen Salloway[14], Bernardino Ghetti[15], John M. Ringman[16], James M. Noble[17], Jasmeer Chhatwal[18], Alison M. Goate[19], Tammie L. S. Benzinger[3], John C. Morris[3], Randall J. Bateman[3], Guoqiao Wang[3], Anne M. Fagan[3], Eric M. McDade[3], Brian A. Gordon[3], Mathias Jucker[1,2]* and Dominantly Inherited Alzheimer Network[20]

Neurofilament light chain (NfL) is a promising fluid biomarker of disease progression for various cerebral proteopathies. Here we leverage the unique characteristics of the Dominantly Inherited Alzheimer Network and ultrasensitive immunoassay technology to demonstrate that NfL levels in the cerebrospinal fluid (n = 187) and serum (n = 405) are correlated with one another and are elevated at the presymptomatic stages of familial Alzheimer's disease. Longitudinal, within-person analysis of serum NfL dynamics (n = 196) confirmed this elevation and further revealed that the rate of change of serum NfL could discriminate mutation carriers from non-mutation carriers almost a decade earlier than cross-sectional absolute NfL levels (that is, 16.2 versus 6.8 years before the estimated symptom onset). Serum NfL rate of change peaked in participants converting from the presymptomatic to the symptomatic stage and was associated with cortical thinning assessed by magnetic resonance imaging, but less so with amyloid-β deposition or glucose metabolism (assessed by positron emission tomography). Serum NfL was predictive for both the rate of cortical thinning and cognitive changes assessed by the Mini-Mental State Examination and Logical Memory test. Thus, NfL dynamics in serum predict disease progression and brain neurodegeneration at the early presymptomatic stages of familial Alzheimer's disease, which supports its potential utility as a clinically useful biomarker.

In most neurodegenerative diseases, brain changes manifest many years before clinical symptoms become apparent. In Alzheimer's disease, presymptomatic changes in the brain include cortical thinning and neuropathological depositions containing amyloid-β and tau. These pathological changes can be assessed by magnetic resonance imaging (MRI), positron-emission tomography (PET), and measurement of amyloid-β and tau protein levels in the cerebrospinal fluid (CSF)[1–4]. However, CSF collection is invasive and imaging modalities are expensive; therefore, they are not well suited to routine clinical practice. Blood biomarkers for the presymptomatic phase of Alzheimer's disease are largely lacking, although recent progress in the analysis of amyloid-β, tau, and neurofilament light chain (NfL) in the blood have been reported[5–10].

NfL is a component of the axonal cytoskeleton and is primarily expressed in large-caliber myelinated axons[11,12]. Changes of NfL in bodily fluids have been linked to brain damage and brain atrophy in mouse models and multiple neurological disorders including

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Fig. 3 | Rate of change per year in serum NfL in mutation carriers mirrors rate of change in cortical thinning. a, Relationship between estimated annual rate of change in serum NfL and estimated annual rate of change in precuneus cortical thickness for non-carriers, presymptomatic (Presym) mutation carriers, and symptomatic (Sym) mutation carriers (including converters to the symptomatic phase, see Fig. 2c). Results from LMEMs revealed

Simoa: NfL predicts clinical progression in pre-symptomatic Alzheimer's disease 16 years before onset

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nature medicine

Oliver Preische1,2,21, Stephanie A. Schultz3,21, Anja Apel1,2,21, Jens Kuhle4, Stephan A. Kaeser1,2, Christian Barro4, Susanne Gräber1, Elke Kuder-Buletta1, Christian LaFougere1, Christoph Laske1,2, Jonathan Vöglein5,6, Johannes Levin5,6, Colin L. Masters7, Ralph Martins8,9, Peter R. Schofield 10,11, Martin N. Rossor12, Neill R. Graff-Radford13, Stephen Salloway14, Bernardino Ghetti 15, John M. Ringman16, James M. Noble 17, Jasmeer Chhatwal18, Alison M. Goate 19, Tammie L. S. Benzinger 3, John C. Morris3, Randall J. Bateman3, Guoqiao Wang3, Anne M. Fagan3, Eric M. McDade3, Brian A. Gordon 3, Mathias Jucker1,2* and Dominantly Inherited Alzheimer Network20

Digital Biomarkers Disruption Paradigm: Alzheimer's Disease Opportunity

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Today

Alzheimer's disease not diagnosed until symptoms

Imaging expensive and often not covered

Therapies for later stage disease have limited effectiveness

Tomorrow

Detect or screen in annual blood test

CNN

Follow on Image or blood test to diagnose

Therapy delivered sooner with less dosing / toxicity. Blood test monitors progression

Aducanumab from Biogen

Taking Aim at Alzheimer's Disease with Simple Blood Test

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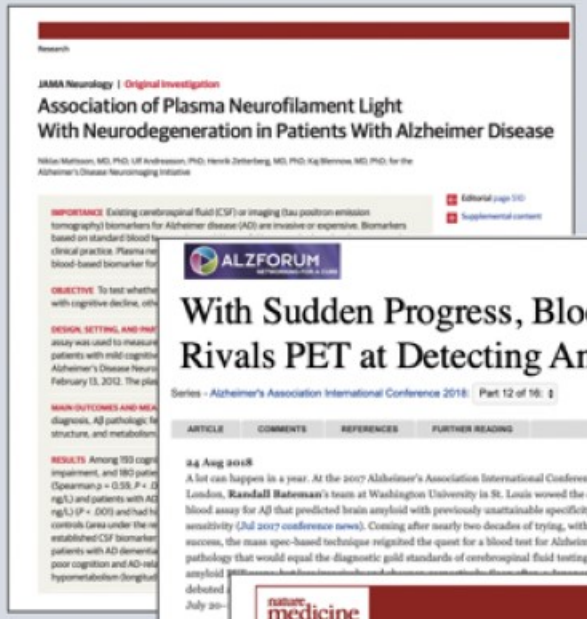
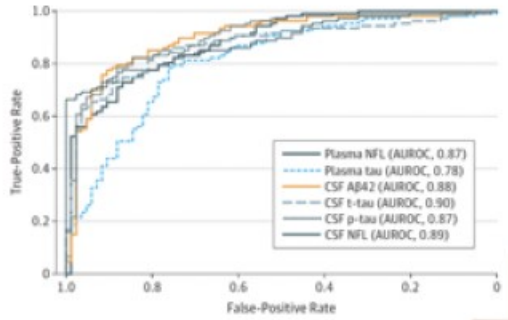

JAMA Neurology | Original Investigation

Association of Plasma Neurofilament Light With Neurodegeneration in Patients With Alzheimer Disease

2018



Plasma NfL exhibits high diagnostic accuracy for Alzheimer's Disease (AUC 0.87)

ALZFORUM

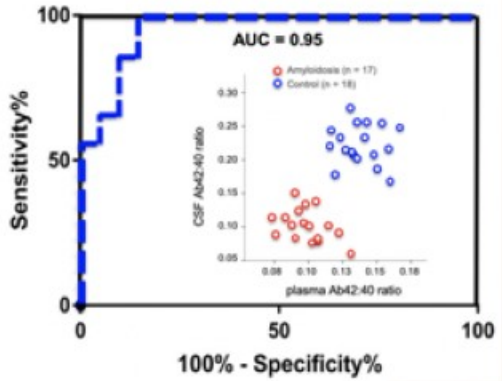
With Sudden Progress, Blood Aβ Rivals PET at Detecting Amyloid

Series - Alzheimer's Association International Conference 2018: Part 12 of 16

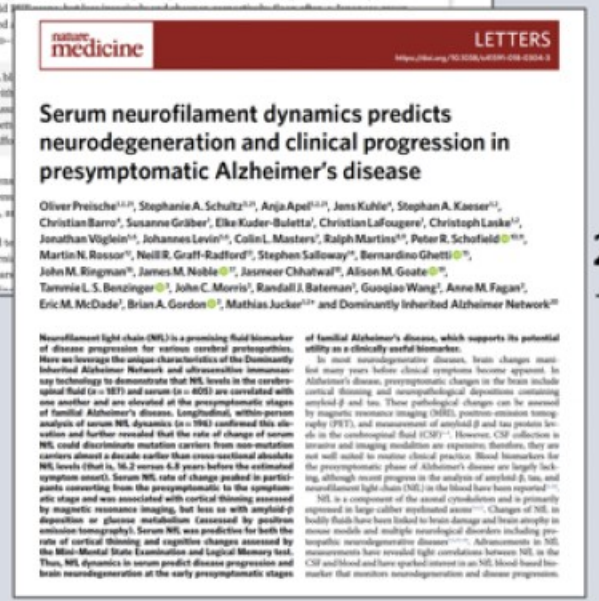
24 Aug 2018

A lot can happen in a year. At the 2017 Alzheimer's Association International Conference in London, **Randall Bateman**'s team at Washington University in St. Louis wowed the crowd with a blood assay for Aβ that predicted brain amyloid with previously unattainable specificity and sensitivity (Jul 2017 conference news). Coming after nearly two decades of trying, without much success, the mass spec-based technique reignited the quest for a blood test for Alzheimer's pathology that would equal the diagnostic gold standards of cerebrospinal fluid testing and

2018



Plasma Aβ42/Aβ40 ratio exhibits high diagnostic accuracy for **amyloid positive patients (AUC 0.95)**


nature medicine LETTERS

Serum neurofilament dynamics predicts neurodegeneration and clinical progression in presymptomatic Alzheimer's disease

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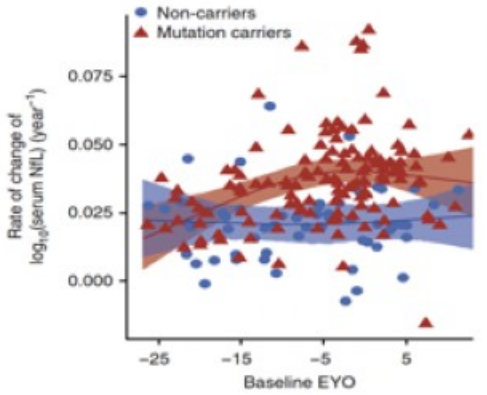
2019



Serum NfL predicts clinical progression in pre-symptomatic Alzheimer's disease **16 years before onset.**

NEW BLOOD TEST CAN PREDICT ALZHEIMER'S DISEASE

LIVE CNN

FOX NEWS

$1,400M

LUND UNIVERSITY

UNIVERSITY OF GOTHENBURG

ADNI Alzheimer's Disease Neuroimaging Initiative

MERCK

AstraZeneca

NOVARTIS

janssen

Amsterdam UMC Universitair Medische Centra

Biogen Aducanumab

Genentech A Member of the Roche Group

Lilly

GRIFOLS

Allergan

DZNE German Center for Neurodegenerative Diseases within the Helmholtz Association

Every **65 SECONDS** SOMEONE IN THE UNITED STATES DEVELOPS THE DISEASE

Every **3 SECONDS** SOMEONE IN THE WORLD DEVELOPS THE DISEASE

5.8 MILLION AMERICANS ARE LIVING WITH ALZHEIMER'S. BY 2050, THIS NUMBER IS PROJECTED TO RISE TO NEARLY 14 MILLION.

44 MILLION PEOPLE ARE LIVING WITH ALZHEIMER'S WORLDWIDE

TRACK-TBI Definitive study on NFL in brain injury - NIH / UCSF

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NIH National Institute of Neurological Disorders and Stroke

Penn University of Pennsylvania

TRACK-TBI Transforming Research and Clinical Knowledge in Traumatic Brain Injury
International Traumatic Brain Injury Research Initiative

Department of Defense

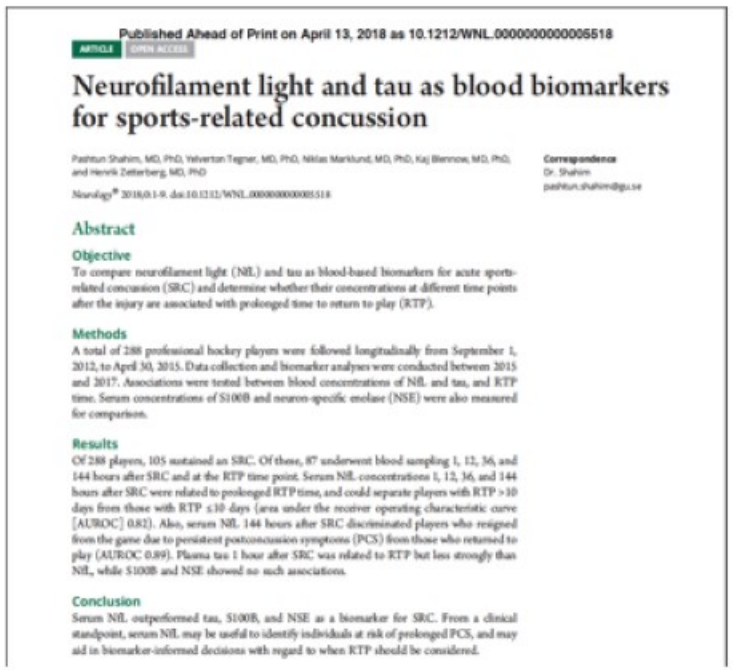
UCSF University of California San Francisco

Statistics Canada

Geoff Manley MD PhD
UCSF, TRACK-TBI

Prospective TBI trial comparing Simoa NFL to imaging and outcome measures with generation of large normative database

- Dr. Ramon Diaz-Arristia, principle investigator; TRACK-TBI P.I. Dr. Geoff Manley
- TRACK-TBI: 18 collaborating US sites, 3,000 longitudinal brain injury patients
- Canadian Health Measures Survey / Statistics Canada, 6,000 healthy subjects
- Framingham Heart Study OMNI cohort – ethnic diversity, 900 subjects
- Supported in part by Quanterix



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ARTICLE OPEN ACCESS

Neurofilament light and tau as blood biomarkers for sports-related concussion

Pashtun Shahim, MD, PhD, Yelverton Tegner, MD, PhD, Niklas Marklund, MD, PhD, Kaj Blennow, MD, PhD, and Henrik Zetterberg, MD, PhD

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Neurology® 2018;0:1-9. doi:10.1212/WNL.0000000000005518

Abstract

Objective
To compare neurofilament light (NfL) and tau as blood-based biomarkers for acute sports-related concussion (SRC) and determine whether their concentrations at different time points after the injury are associated with prolonged time to return to play (RTP).

Methods
A total of 288 professional hockey players were followed longitudinally from September 1, 2012, to April 30, 2015. Data collection and biomarker analyses were conducted between 2015 and 2017. Associations were tested between blood concentrations of NfL and tau, and RTP time. Serum concentrations of S100B and neuron-specific enolase (NSE) were also measured for comparison.

Results
Of 288 players, 105 sustained an SRC. Of these, 87 underwent blood sampling 1, 12, 36, and 144 hours after SRC and at the RTP time point. Serum NfL concentrations 1, 12, 36, and 144 hours after SRC were related to prolonged RTP time, and could separate players with RTP >10 days from those with RTP ≤10 days (area under the receiver operating characteristic curve [AUROC] 0.82). Also, serum NfL 144 hours after SRC discriminated players who resigned from the game due to persistent postconcussion symptoms (PCS) from those who returned to play (AUROC 0.89). Plasma tau 1 hour after SRC was related to RTP but less strongly than NfL, while S100B and NSE showed no such associations.

Conclusion
Serum NfL outperformed tau, S100B, and NSE as a biomarker for SRC. From a clinical standpoint, serum NfL may be useful to identify individuals at risk of prolonged PCS, and may aid in biomarker-informed decisions with regard to when RTP should be considered.

Ultrasensitive LIF Assay Revealed as Promising Blood Biomarker Drug Target for Pancreatic Cancer

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Science News *from research organizations*

New study targets Achilles' heel of pancreatic cancer, with promising results

Researchers discover a potential therapeutic target for deadly cancer

Date: April 17, 2019

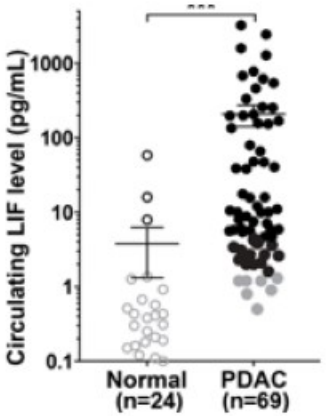
Source: Salk Institute

Summary: Advanced pancreatic cancer is often symptomless, leading to late diagnosis only after metastases have spread throughout the body. Now, researchers have uncovered the role of a signaling protein, called LIF, that may be the Achilles' heel of pancreatic cancer.

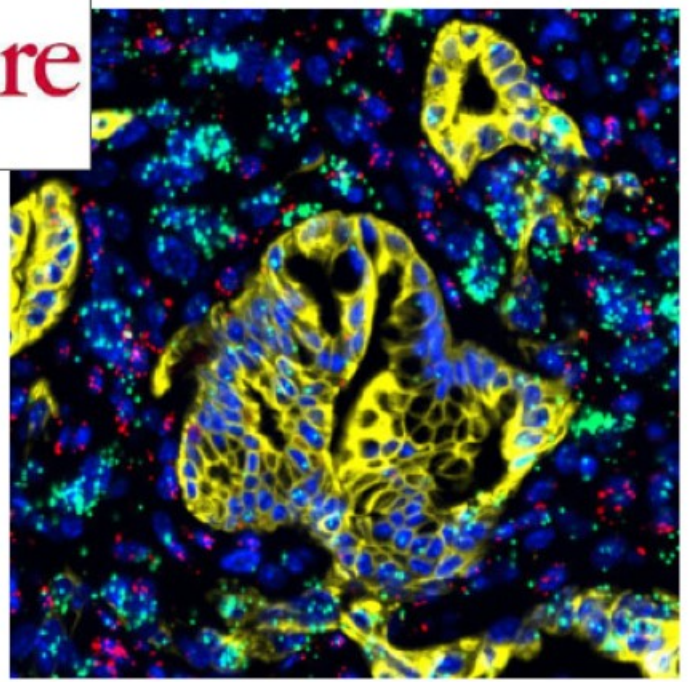
Simoa LIF assay is more sensitive than Luminex

		Luminex				Simoa			
		LIF(pg/mL)	FI-1	FI-2	Avg. FI	LIF(pg/mL)	AEB-1	AEB-2	Avg. AEB
Standard Curve	Blank	0	64	70	67	0	0.0143	0.0133	0.0138
	S1	6	87	78	82	0.11	0.0252	0.0200	0.0226
	S2	24	141	132	137	0.44	0.0420	0.0403	0.0411
	S3	98	323	303	313	1.76	0.1017	0.1121	0.1069
	S4	391	914	903	908	7.03	0.3762	0.3867	0.3815
	S5	1563	2745	2802	2773	28.13	1.5377	1.4952	1.5164
	S6	6250	7260	7129	7194	112.50	5.4800	5.6538	5.5669
	S7	25000	12974	12668	12821	450.00	21.3583	20.4018	20.8801

LOD: 0.048 pg/mL



nature



Credit: Salk Institute

SALK INSTITUTE
FOR BIOLOGICAL STUDIES

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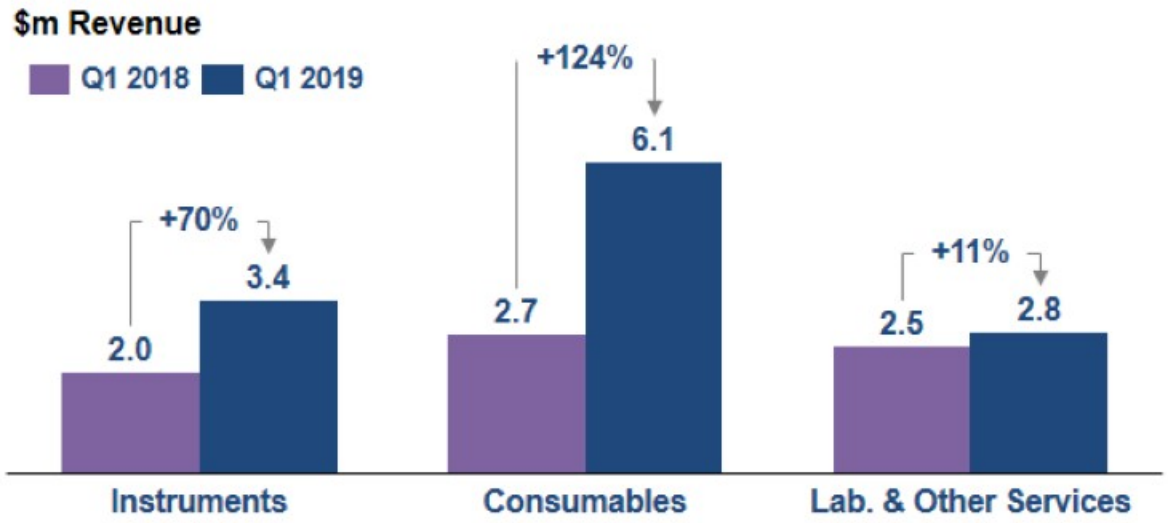
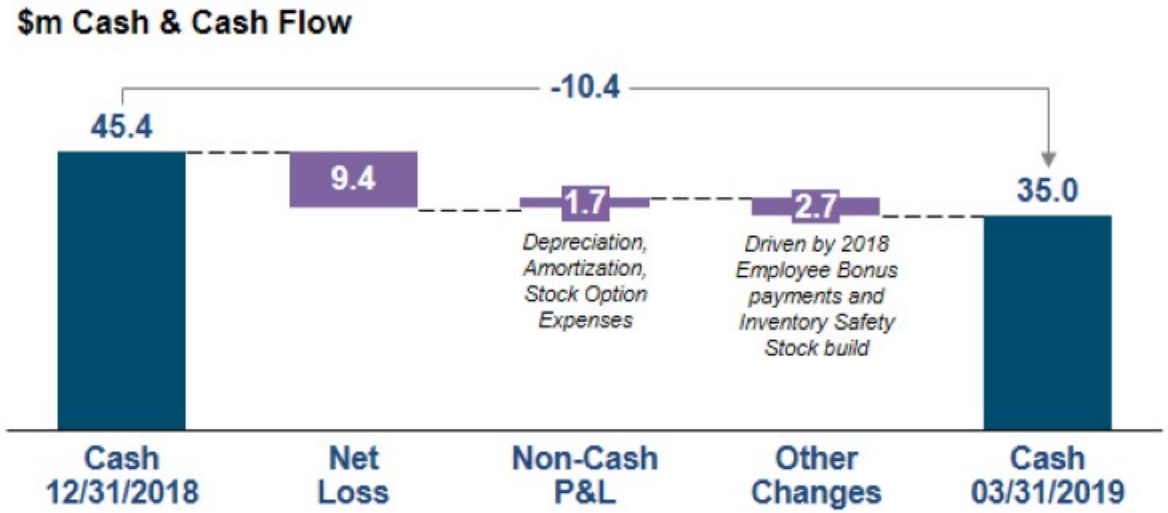
in $m	Actuals	Prior Year	
	Q1 2019	Q1 2018	Var.
Product Revenue	**9.5**	4.7	+4.8
Growth vs. PYR	***+101%***		
Lab/Services	**2.8**	2.5	+0.3
Growth vs. PYR	***+11%***		
Collaboration	**-**	0.3	-0.3
Total Revenue	**12.3**	**7.5**	**+4.8**
Growth vs. PYR	***+64%***		
Gross Profit	**6.0**	**3.2**	**+2.8**
Gross Margin %	***48.7%***	*42.2%*	*+650 bps*
Operating Expenses	**15.4**	10.3	+5.0
Loss from operations	**-9.4**	**-7.2**	**-2.2**





Poised to Disrupt Healthcare and Create Significant Value

Quanterix
The Science of Precision Health

market

execution

Quanterix

		Differentiator	Value
1	Category-defining; Unrivaled Sensitivity / Technology	Best in Class	Disrupt
2	Methodical market penetration strategy to reward investors	$3B to $38B	New Answers
3	DNA – RNA - Protein; Better linked to Disease / Health		Holy Grail
4	**Validation**: 19/20 top pharma, PPH, 800+ trials	500+ pubs All Areas	Proven
5	Growth & Value; Razor – razor blade, $150M invested	Product Launches	Rapid Growth
6	Low Risk / Solid Return + Uber Return Prospect		Retail
7	Track Record for Commercializing Disruption		Lynchpin

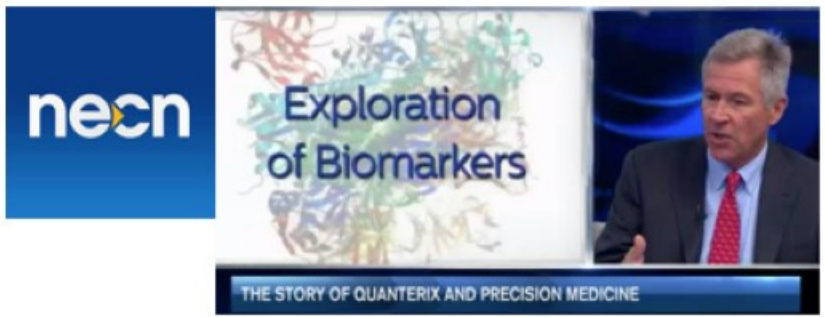
Quanterix in the News – Q1 2019

Quanterix
The Science of Precision Health

Quanterix continued to build awareness for its mission to advance the science of precision health in mainstream press and trade media.

PPH 2018 - Amsterdam

MEDIA HIGHLIGHTS



The Story of Quanterix and Precision Medicine

Hrusovsky believes that in order to succeed, a business must change the world in a positive way. As he said, "We believe that by powering precision health, we are helping to eradicate the causes of Alzheimer's, ALS, and Parkinson's disease."

Forbes

ABC – Feb 2019

Bloomberg

genomeweb

Alzheimer's Might be Detectable With Biomarker Test 16 Years Before Onset of Symptoms

BOSTON BUSINESS JOURNAL

CNN

USA TODAY

Bloomberg Radio Feb 2019

New technology could detect cancer in blood before symptoms develop

HUFFPOST

MULTIPLE SCLEROSIS NEWS TODAY

Diagnostics World
Beyond the Odyssey: Emerging Technologies in Diagnostics

Neurofilament Light Chain Levels in Blood of Value as Biomarker of MS Activity and Treatment Response, Study Finds