

# A new standard in cholesterol therapy



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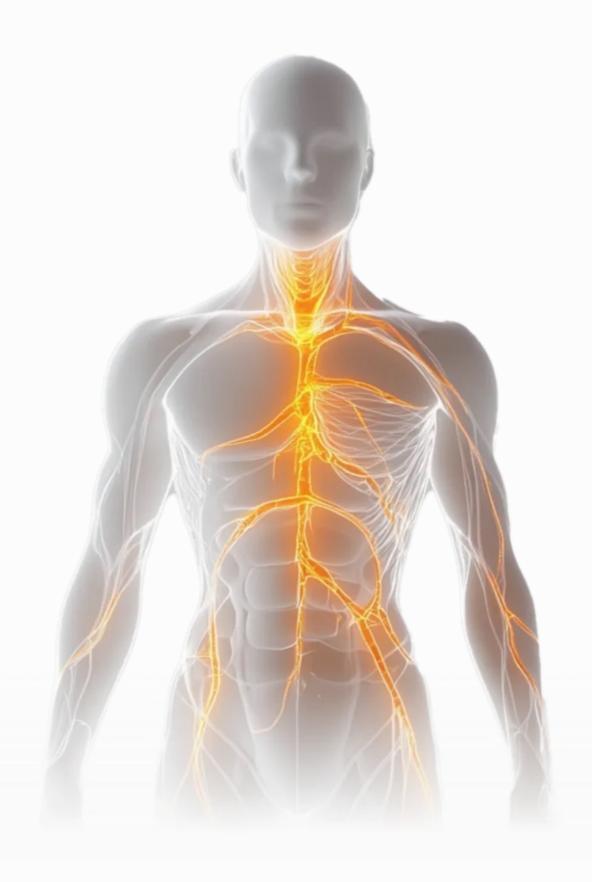
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# About Aqur

A pioneering bioscience company headquartered in Westlake Village, California, United States



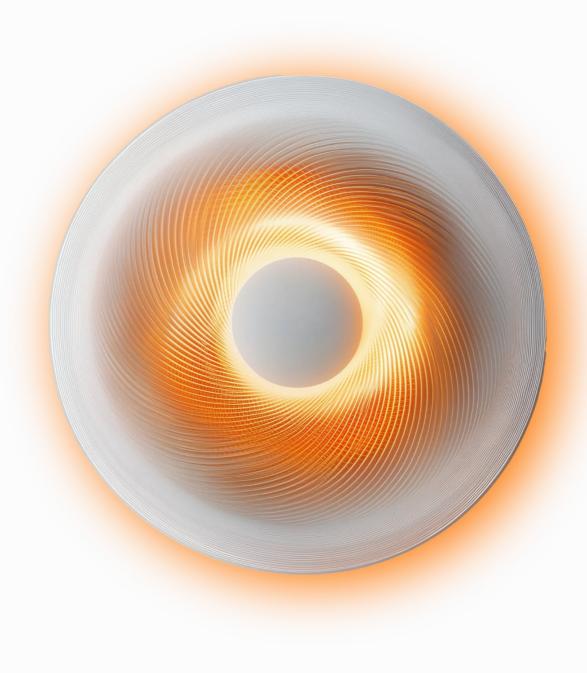
Developing a revolutionary oral therapy targeting high cholesterol, addressing a \$25+ billion market



# **Core Values**

### Vision

Pioneering a future where cardiovascular disease caused by high cholesterol is effectively treated with cutting-edge, and patient-friendly therapies.



### Team

Our team consists of world-renowned scientists, researchers, and industry experts with deep expertise in bioscience, pharmaceutical discovery, and cardiovascular health. Collectively, the team has contributed to the development of over 30 commercial drugs, generating more than \$10 billion in sales.

### Mission

Dedicated to advancing breakthrough treatments that address some of the most urgent health challenges, ensuring safety, efficacy, affordability, and accessibility.



# High Cholesterol & Cardiovascular Disease



### CVD

High cholesterol is a major contributing factor to the Cardiovascular Disease (CVD)

### #1

CVD is the number 1 cause of death worldwide, responsible for ~31% of all annual global deaths

### >1 billion

People suffer from high cholesterol globally, affecting approximately ~39% of adults (~94M Americans)

### 18.6 million

Annual cardiovascular deaths, with ~ 85% attributable to atherosclerotic CVD, primarily caused by elevated LDL



# **Current Cholesterol Therapeutics: Statins**



### MoA

Competitive inhibition of enzyme HMG-CoA reductase

### Administration

Oral

### Effect

Reduces cholesterol level & helps stabilize atherosclerotic plaques

3 / Current Therapeutics



### LDL

Typical reduction: 30 – 50%

Cost Low

### Side Effects

Muscle-related & gastrointestinal symptoms, liver enzyme elevation (reversible & rare)



# **Current Cholesterol Therapeutics: PCSK9i**



### MoA

mAbs - binds to PCSK9 siRNA - interfere with PCSK9 synthesis

### Administration

Injectable

### Effect

Significantly reduces LDL cholesterol levels in high-risk patients 3 / Current Therapeutics



### LDL

Typical reduction: 50 - 70%

Cost High

### Side Effects

Injection site reactions, flu-like symptoms, hypersensitivity or allergic reactions (rare)



# **Current Market**

**U**NOVARTIS · 2 Alnylam

SANOFI 🌍 REGENERON

### Statins (*incl. generics*): ~ \$16 Billion



**AMGEN** 

2020 - Novartis acquired The Medicines Company for \$9.7B, primarily for inclisiran (Leqvio), then under-development

2020 - AstraZeneca acquired Dogma Therapeutics' oral PCSK9 inhibitor, for an undisclosed amount

### PCSK9i injectables: ~ \$2.7 Billion





~\$754M Sales in 2024

~\$725M Sales in 2024



~\$1.22B Sales in 2024



# **Therapies Under Development**

**Targeting PCSK9** 

SUPPRESSED PCSK9



AZD0780 CLINICAL RESEARCH PHASE II



MK-0616 CLINICAL RESEARCH PHASE III

### **Targeting LDL-R**

#### PCSK9 UNAFFECTED



#### AQR-008 PRE-CLINICAL RESEARCH



# Aqur's Oral PCSK9i

# Portfolio of over 50 peptides derived from PCSK9's catalytic domain

Patent granted in 2020



Leading candidate

Royalty free

### Pre-IND stage



### AQR-008: MoA



### Targets

EGF-A domain on the Low-Density Lipoprotein Receptors (LDL-R)

### Prevents

PCSK9 binding to LDL-R, process that leads to LDL-R degradation in the hepatic cell

### Allows

LDL-R recycling back to the hepatic cell membrane, and recurrent binding to LDL particles for clearance



# AQR-008: Advantages

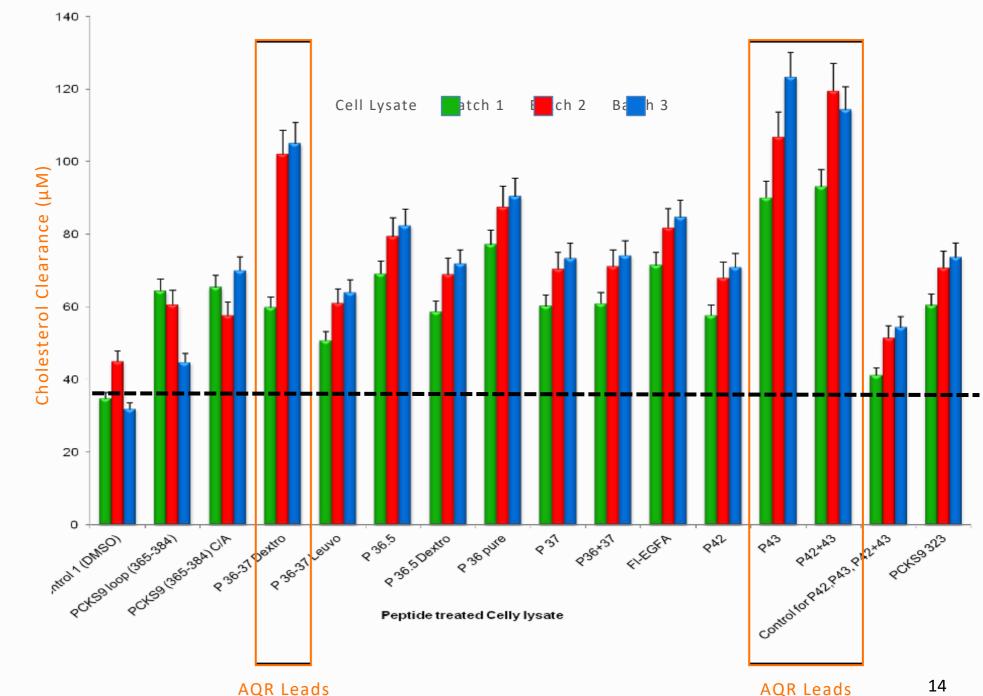




- Minimizes side effects
- Affordable for patients
- Improved patient compliance
- Storage without refrigeration

# **Preliminary Cholesterol Clearance Results**

Aqur's peptides exhibited nearly **3x Cholesterol Clearance** 

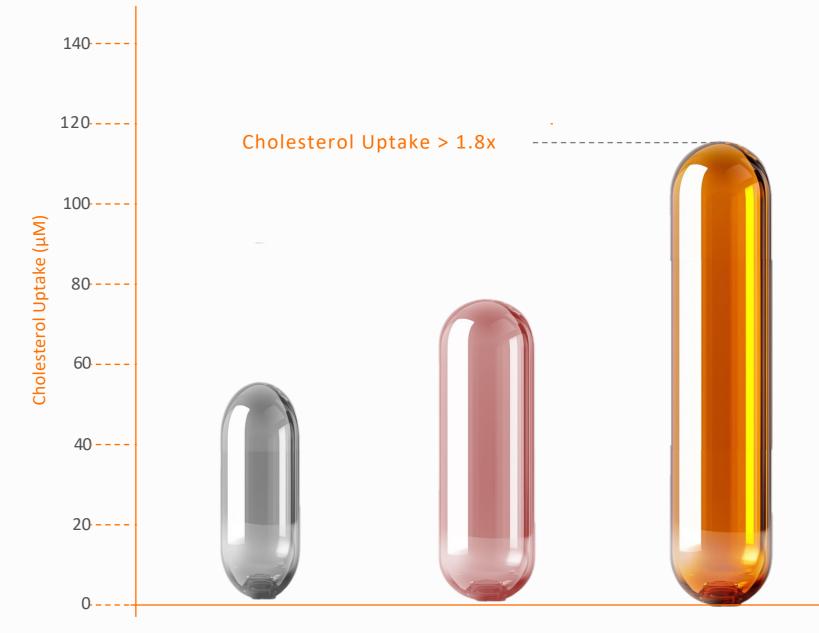


AQR Leads



# **Preliminary Cholesterol Uptake Results**

Aqur's leading peptides increase Cholesterol Uptake levels (>1.8x) in HepG2 cells



Control

#### 6 / Preliminary Results

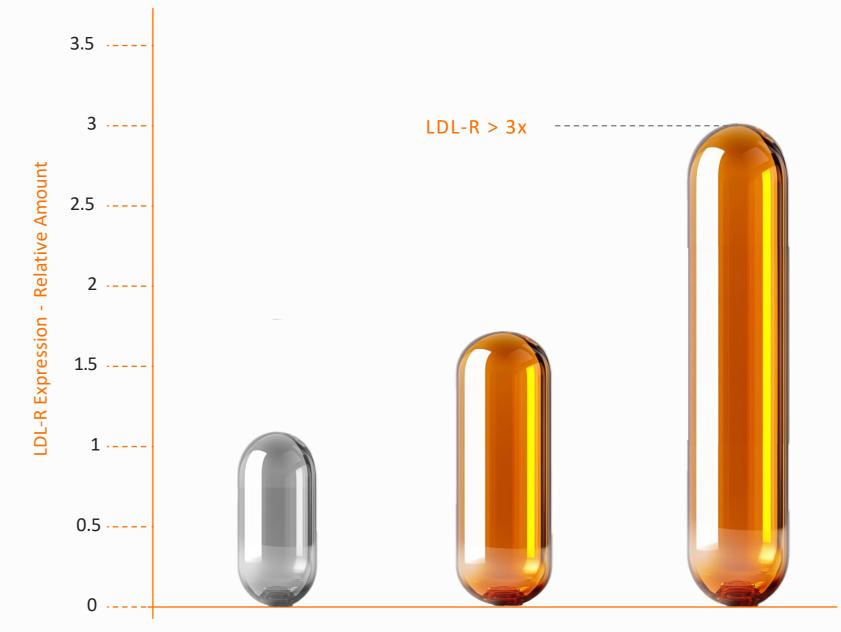
50 µM AQR-007

50 µM AQR-008



# AQR-008: Pre-Clinical Efficacy

Significantly enhanced (>3x) LDL-R Expression in human hepatic cells at  $50 \mu M$  concentration



Control

#### 6 / Preliminary Results

5 μΜ AQR-008

50 µM AQR-008

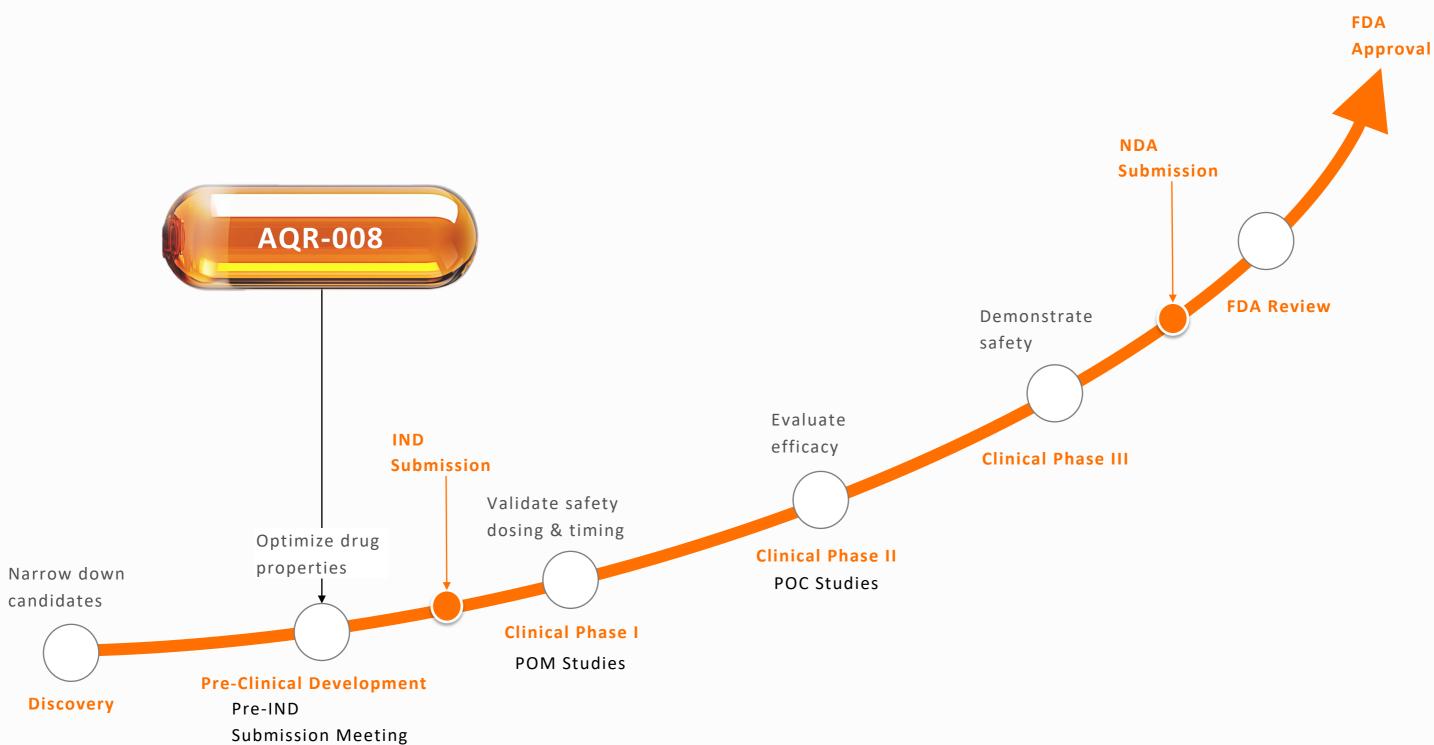


# AQR-008 Key Points

STAGE	In pre-clinical studies			
	Targets the EFG-A domain on the LDL-R			
MECHANISM OF ACTION	Blocks LDL-R from binding with PCSK9			
	Prevents LDL-R degradation & enables its recycling			
DELIVERY METHOD	Oral			
RESULTS & EFFECTS	Significant LDL cholesterol reduction			
	PCSK9 UNAFFECTED			
DCCKO	Does NOT bind to PCSK9			
PCSK9	Does NOT suppress PCSK9 production			
	Does NOT interfere with other PCSK9 functions			
SIDE EFFECTS	Minimal side effects			
COMPLIANCE	High compliance due to oral delivery, low cost & minimal side effects			
COST	Designed for patient affordability			

7 / Key Points

# Path Forward





### **Strategic Partners**

# charles river = QVIA PEPTIC





8 / Partners







# **Professionals**

# **McGuireWoods**







### 

# **Management Team**



#### Michael A. Fole, MD, MBA Chief Executive Officer

25+ years of experience in wealth & family office management, banking, trading and investments

MD - University of Medicine & Pharmacy Carol Davila, Bucharest

MBA - University of La Verne, California



#### Jeffrey B. Wolin, JD President

25+ years investment banking, corporate transactions, mergers & acquisitions, public filings and financings

BS - Biomedical and Chemical Engineering - Columbia University



#### Andy Jennings, PhD Chief Scientific Officer

35+ years of pharma and biotech industry (GSK, Takeda, & Structure Therapeutics)

Published in Computational Chemistry, Medicinal Chemistry, Structural Biology, Assay Development, and Bioinformatics

BSc - Medicinal Chemistry (University of Hertfordshire) and PhD - Computational Chemistry & Bioinformatics - The Open University - UK



### James Hess Leach Chief Financial Officer

40+ years of experience in family office venture capital

Chairman & Senior Managing Director, National Trust, LLC

Chair Emeritus, Rhode Island PBS Foundation & Inductee, Rhode Island Heritage Hall of Fame

#### 9/Team



#### Michel F. Denarie, MBA Chief Operating Officer

37+ years veteran of the pharmaceutical industry

21 years with IQVIA - Senior Principal, Strategic Drug Development - Global COE Lead, Patient Insights - Senior Principal, Commercial Effectiveness

BS - Kogod School of Business - American University

MBA - Darden School of Business - University of Virgina



# **Board of Directors**



#### Jeffrey B. Wolin, JD

25+ years investment banking, corporate transactions, mergers & acquisitions, public filings and financings

BS - Biomedical and Chemical Engineering - Columbia University



#### James Hess Leach

40+ years of experience in family office venture capital Chairman & Senior Managing Director, National Trust, LLC

Chair Emeritus, Rhode Island PBS Foundation & Inductee, Rhode Island Heritage Hall of Fame



#### Madison F. Richardson, MD

40+ years board-certified surgeon

Officer, Medical Board of California (1987-1993)

Former Asst. Professor of Surgery, USC & UCLA Medical Schools

Lt. Col., Walter Reed National Military Medical Center (1968-1979)



#### Valerie Ferguson

40+ years in management

Former Regional General Manager – Deluxe Resorts at Walt Disney World

Former Chairman of the American Hotel & Lodging Association (AHGLA)

Held senior roles at Loews Hotels Corporation & The Ritz-Carlton Hotel Company

#### 9/Team



#### Amanuel Sima, MD

24+ years board-certified physician

Board-certified physician specializing in internal medicine

Diplomate of the American Board of Internal Medicine, the American Board of Pulmonary Medicine, & the American Board of Critical Care Medicine

# Scientific Advisory Board



#### Roland Wandeler, PhD

15+ years of commercial leadership and general management experience in the pharmaceutical & biotechnology industry

Former Amgen General Manager Germany in Spain & Portugal, and Corporate Vice President & General Manager of Amgen's US Bone Health & Cardiology Business Unit

MSc & PhD - Chemical Engineering - ETH Zurich



#### Wayne Paterson

Director & CEO, Anteris Technologies Ltd

Former Head of Pharmaceuticals (South Korea) & Head of Commercial Operations (China), Roche Pharmaceuticals

President, Merck KGaA Europe, Canada & Australia & Global Head of Cardiovascular Medicine, Merck KGaA (2010-2012)



#### Tomi Sawyer, PhD

Inventor of MCR1 agonist NDP-MSH (Scenesse<sup>®</sup> by Clinuvel) recently approved by the FDA

Invented ALRN-6924 (Aileron) and Iclusig<sup>®</sup> (Ariad/Takeda)

Former titles: Distinguished Scientist (Merck), CSO (Aileron), SVP (Ariad) and Sr. Director (Pfizer)

Past President of the American Peptide Society



#### Nabil Seidah, MC, PhD, OQ, PRSC

Discovered and cloned seven (PC1, PC2, PC4, PC5, PC7, SKI-1 and PCSK9) of the nine known enzymes belonging to the convertase family

Author of over 720 peer-review articles & manuscripts

Recipient of Medical Research Council Scientist Award, McLaughlin Medal of the Royal Society of Canada, the Parizeau Prize of the Association Canadienne-Française, the Pfizer Distinguished Cardiovascular-Metabolic Research Jean-Davignon Award, the Queen Elizabeth II Diamond Jubilee Medal, the "Jacques Genest" Lecturer Award

#### 9/Team



### Jay Horton, MD

Dr. Horton's characterization of PCSK9 & the protein's interactions with LDL-R provided the foundation for the development of PCSK9i

Professor of Internal Medicine and Molecular Genetics, Chief of the Division of Digestive & Liver Diseases

Holds the Robert C. & Veronica Atkin Chair in Obesity & Diabetes Research - University of Texas Southwestern Medical Center at Dallas



### Michael D. Shapiro, DO

Professor of Cardiology and Molecular Medicine at Wake Forest University

Published extensively in the areas of atherosclerosis imaging, lipid disorders, and preventive cardiology with research focuses on PCSK9 physiology and its impact on lipoprotein metabolism

DO - Rowan University School of Osteopathic Medicine

# 

# Scientific Advisory Board



#### Nicola Ferri, PhD

Professor in Pharmacology, Department of Medicine, University of Padua, Italy

Author of >190 peer-review articles & manuscripts, including "Emerging oral therapeutic strategies for inhibiting PCSK9" published in Atherosclerosis Plus

PhD in "Experimental Pharmacology" & PhD in "Toxicology of Environment and Nutrition" - University of Milan, Italy



### Ajoy Basak, MSc, PhD, FIC

Inventor of AQR-008

25+ years of discovery and development of PCSK9, cholesterol regulation & CVD

Adjunct Professor, University of Ottawa, & Affiliate Investigator, Ottawa Hospital Research Institute

Published 150+ peer reviewed articles on PCSK9 enzymes



#### Bruce Auerbach, MS

20+ years of experience in pharmaceutical drug discovery & early clinical development

Former associate Research Fellow at Pfizer specializing in drug discovery & early clinical development, with a concentration in dyslipidemia and metabolic diseases

MS in Pathology and Comparative Medicine - Bowman Gray School of Medicine - Wake Forest University



#### Mary G. Sorci-Thomas, PhD

Professor of Medicine in the Division of Endocrinology, Metabolism & Clinical Nutrition- Medical School of Wisconsin

Funded for 33+ years by the National Institutes of Health & for 20 years participated as a Project Leader on an NIH funded Program Project that studied Atherosclerosis and Lipid Metabolism

PhD - Wake Forest University School of Medicine

#### 9/Team



### David M. Lubman, PhD

Professor Emeritus – University of Michigan Medical School Director, The Lubman Lab (University of Michigan Medical School) investigating novel biotechnologies

MS - Columbia University

PhD - Stanford University



#### Robert E. Burrier, PhD

Career technology executive with experience in pharmaceutical research

Participated & led programs resulting in drugs currently on the market including Zetia & Vytorin

RGD at Schering-Plough, Merck KGaA, Eli Lilly, GelTex, and Genzyme

PhD - Boston University, Postdoctoral Fellowship in Comparative Medicine - Wake Forest University



# At a Glance

### Innovation

**Unique Mechanism of Action** 

Strategy

**Proven Development Pathway** 

### 10 / At a Glance

### **Target** Large Addressable Market

### Advantage

biosciences

Oral, Affordable, Accessible



# Thank You!

# We look forward to the journey ahead, where we can turn our vision into reality.



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