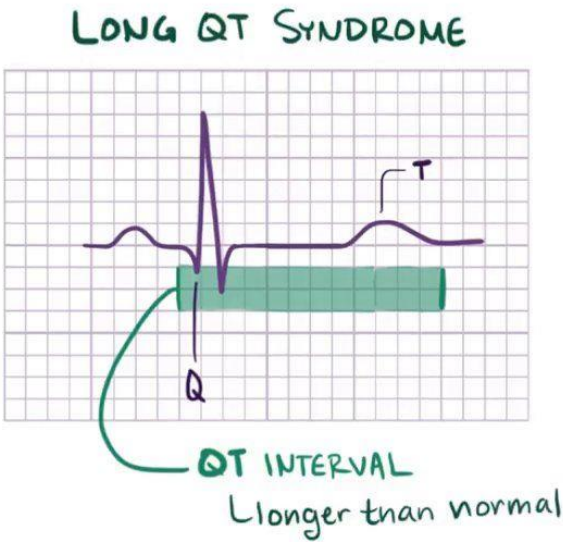
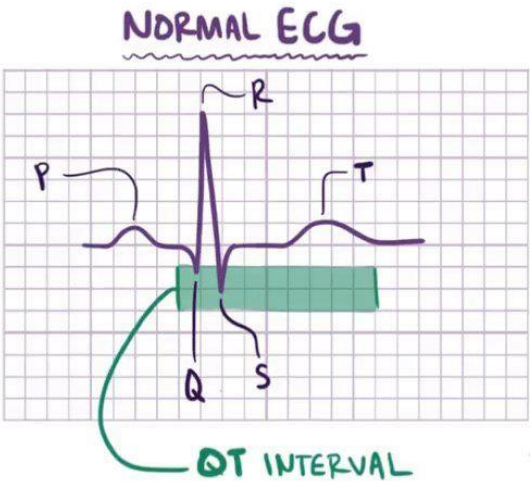


# GENE THERAPY

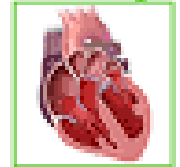
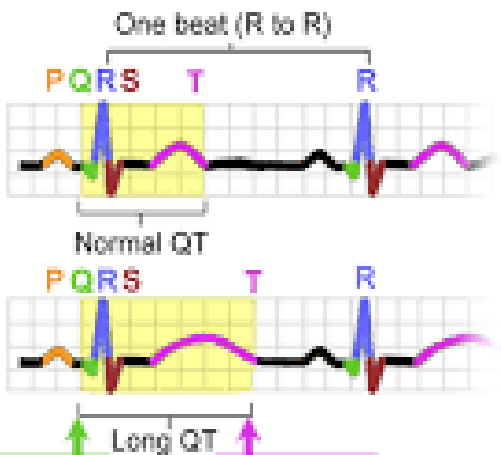
WHAT'S NEW?



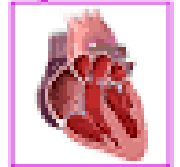
# LONG QT SYNDROME



Each section of an electrocardiogram (ECG or EKG) is referred to by a letter name: Q, R, S, and T.

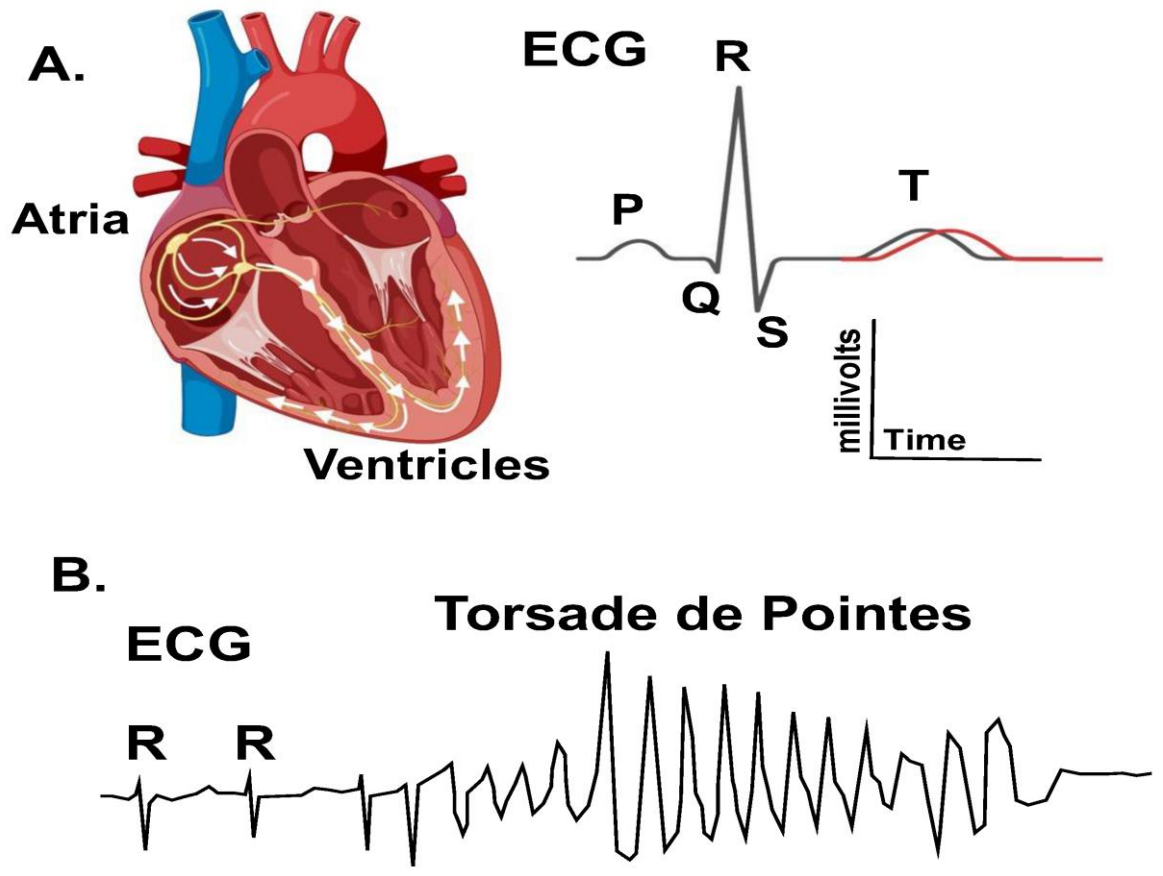


Heart is relaxed and full of blood at Q.



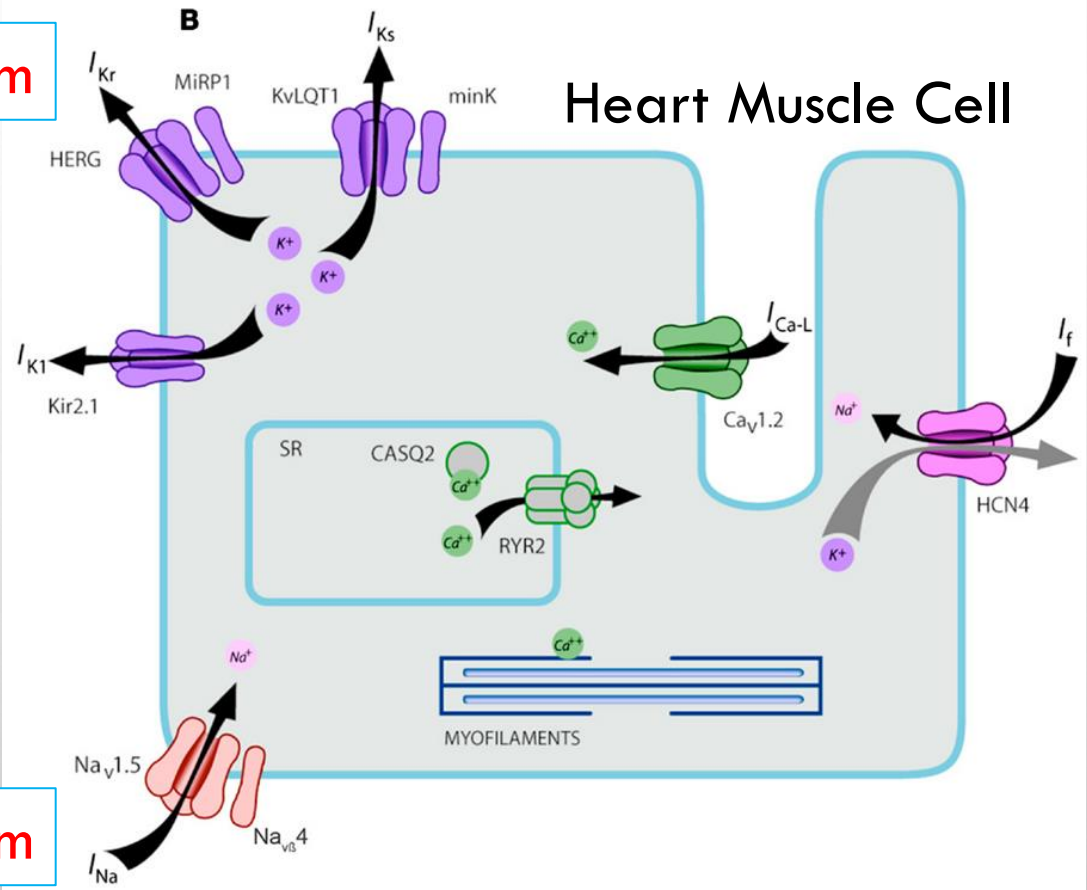
Heart is contracted at end of T.

# LONG QT SYNDROME



# LONG QT SYNDROME

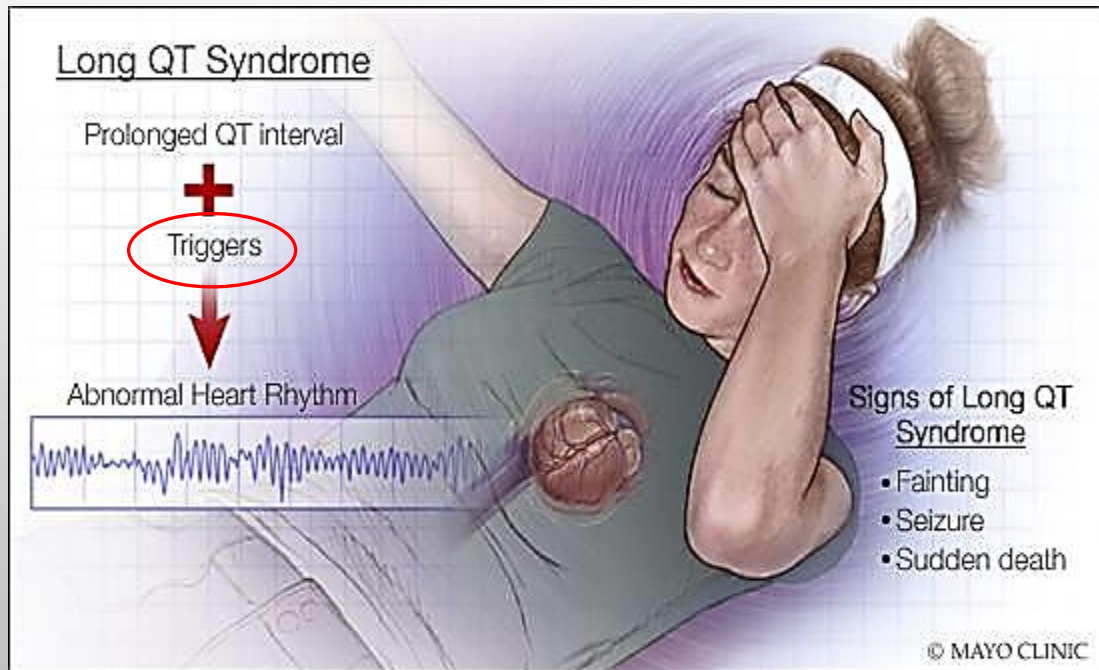
Potassium



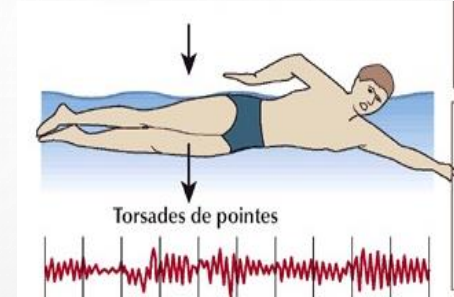
Sodium



# LONG QT TRIGGERS



Type 1



Swimming  
Exercise  
Stress

Type 2



Alarms  
Sudden startle  
Post-partum

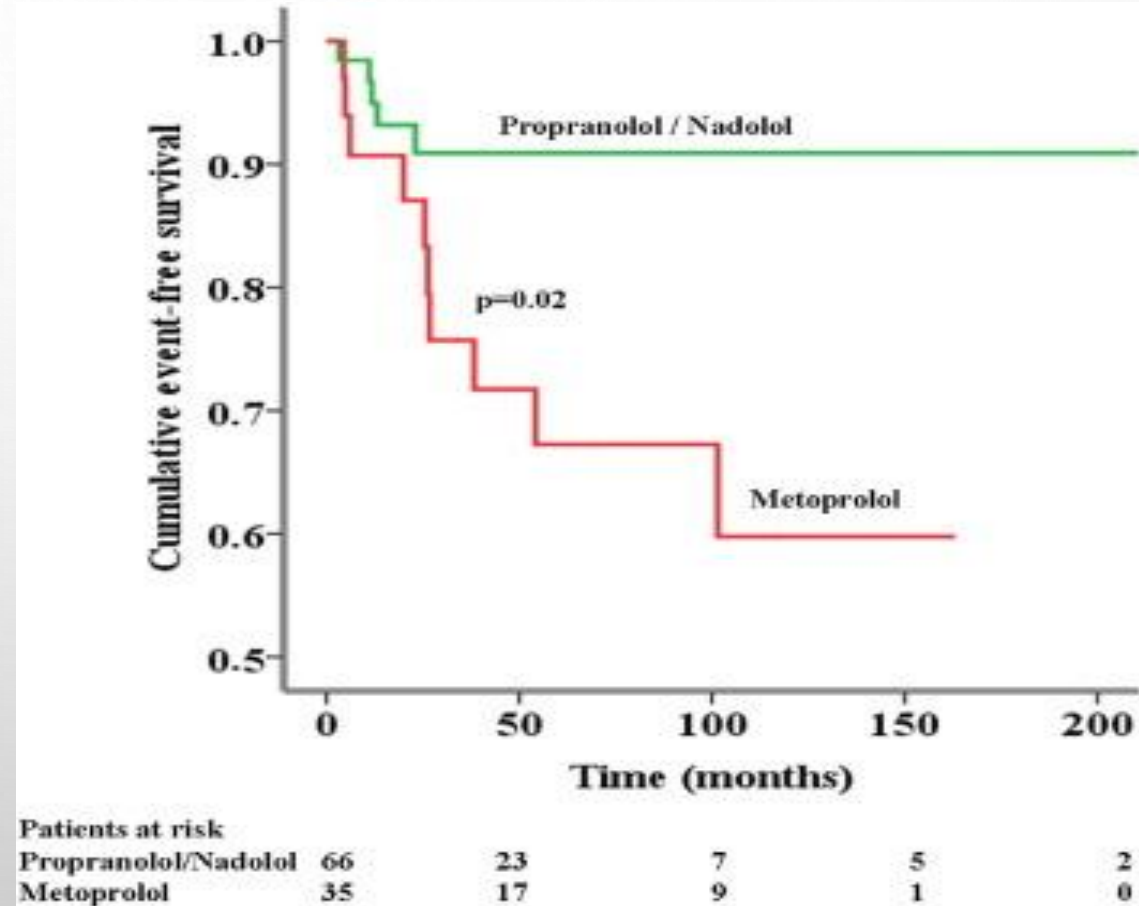
Type 3



Symptoms  
occur  
at rest

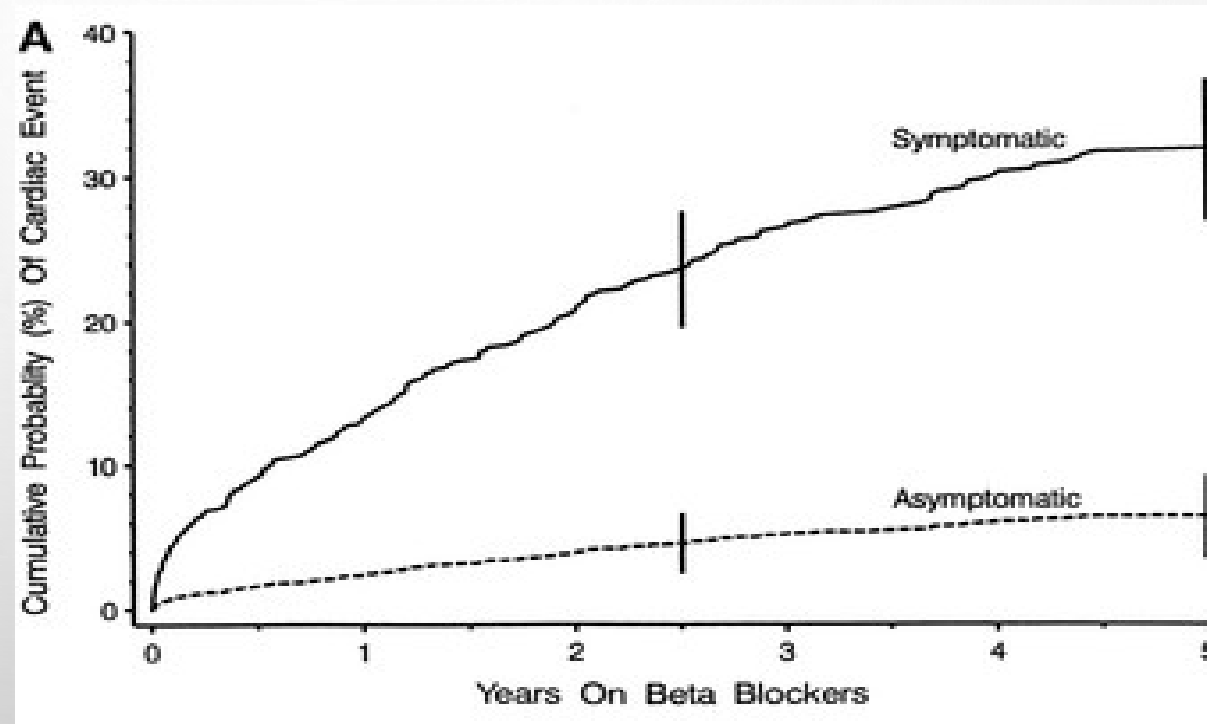
# LQTS TREATMENT

## Beta-Blockers



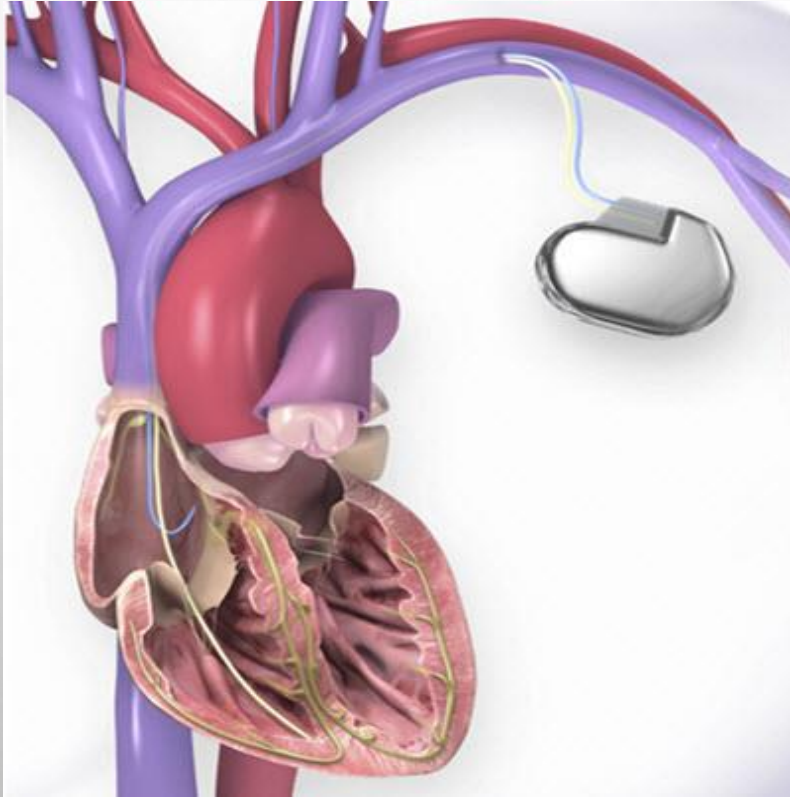
# LQTS TREATMENT

## Beta-Blockers

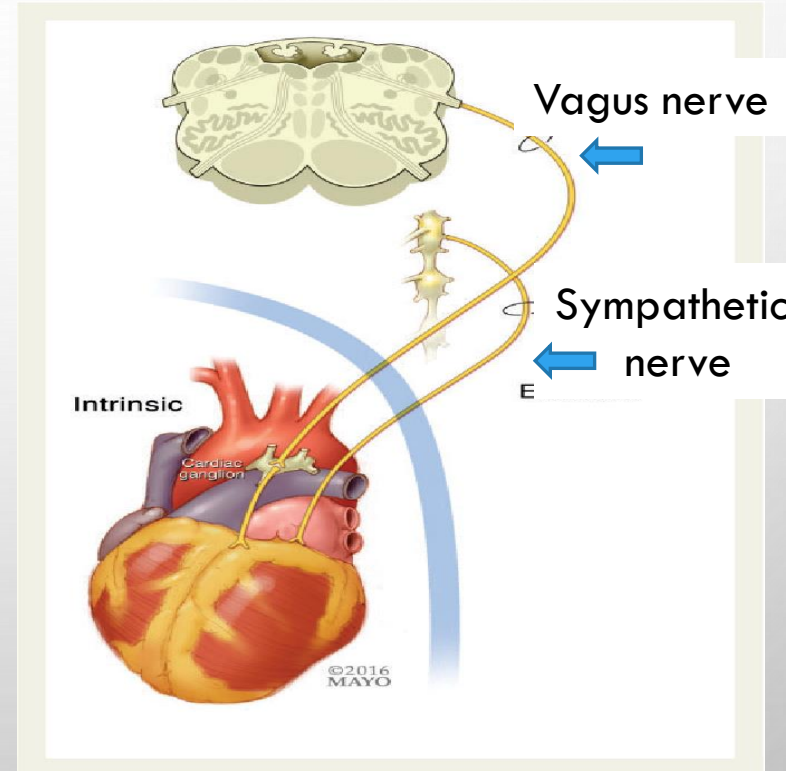


# LQTS TREATMENT

## Pacemakers/ Defibrillators



## Cardiac Sympathectomy

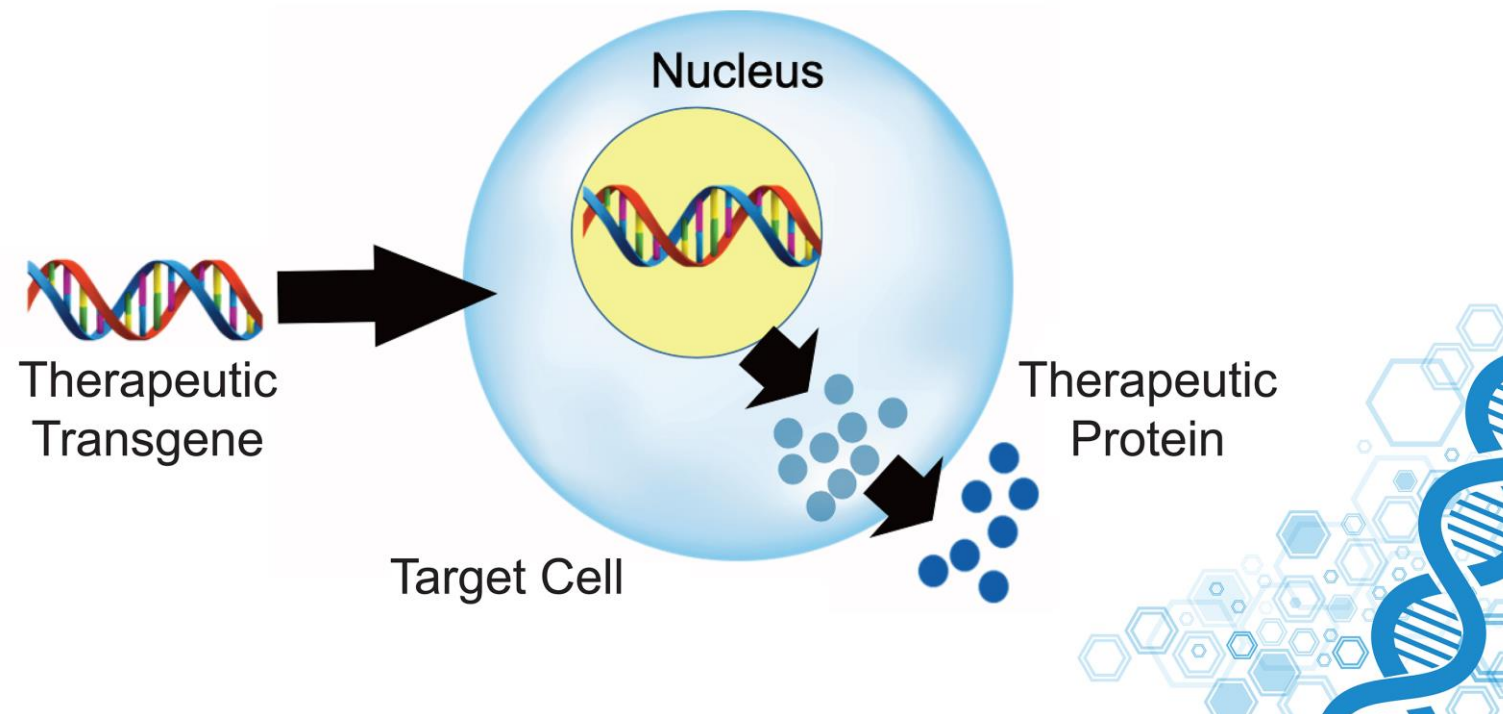




# GENE THERAPY

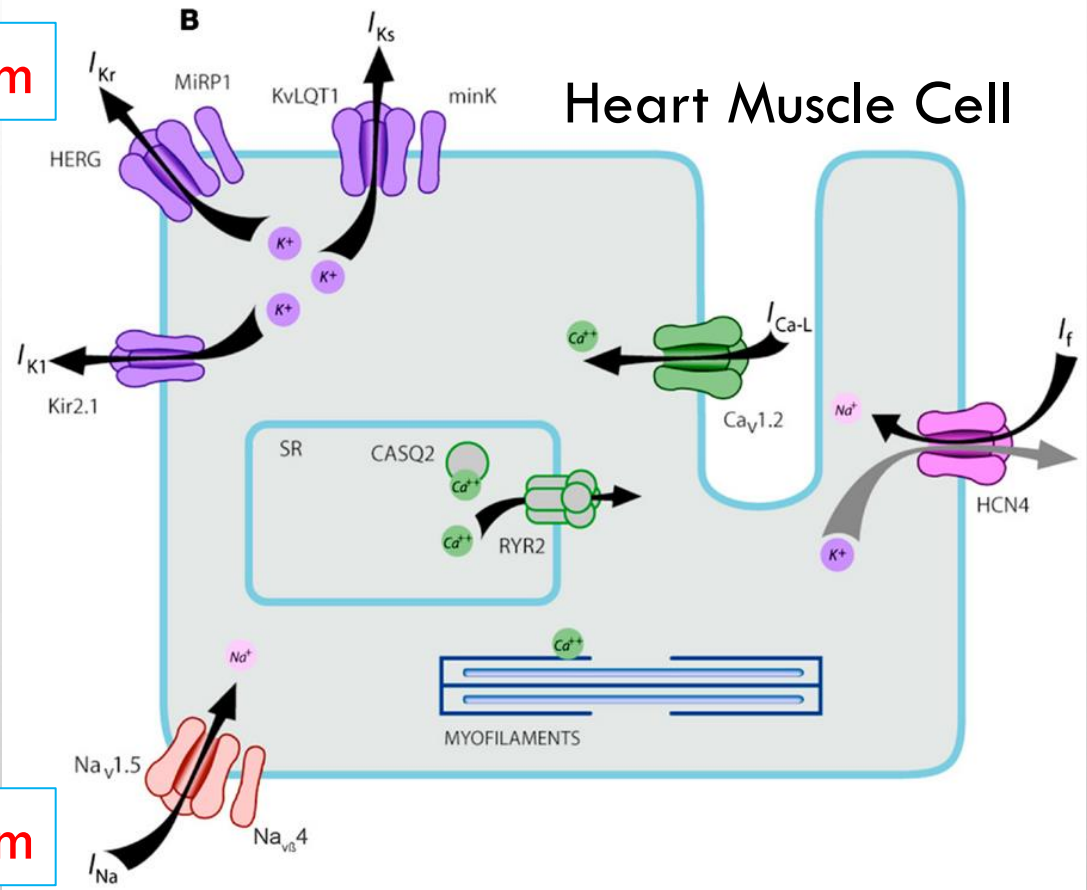


## The Principle of Gene Therapy



# LONG QT SYNDROME

Potassium



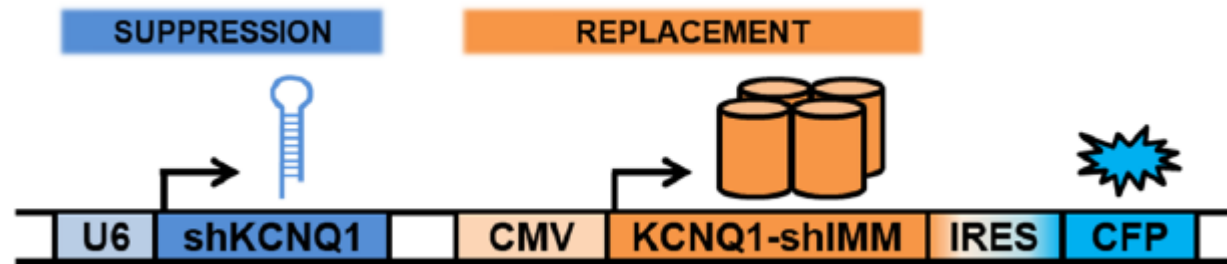
Sodium

## Circulation

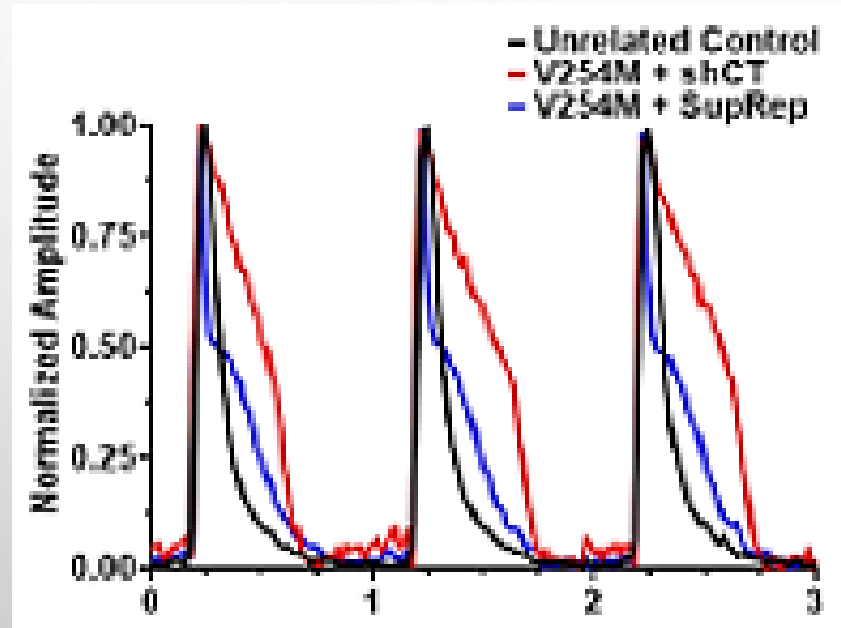
### ORIGINAL RESEARCH ARTICLE

# Suppression-Replacement *KCNQ1* Gene Therapy for Type 1 Long QT Syndrome

B



# GENE THERAPY FOR LQTS 1



## Conclusion

This study provides the first proof-of-principle gene therapy for complete correction of long QT syndrome.

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41.4.5 - Ion Channels, Electrophysiology

**In vivo KCNQ1-suppression-replacement gene therapy in transgenic rabbits with type 1 long QT syndrome**



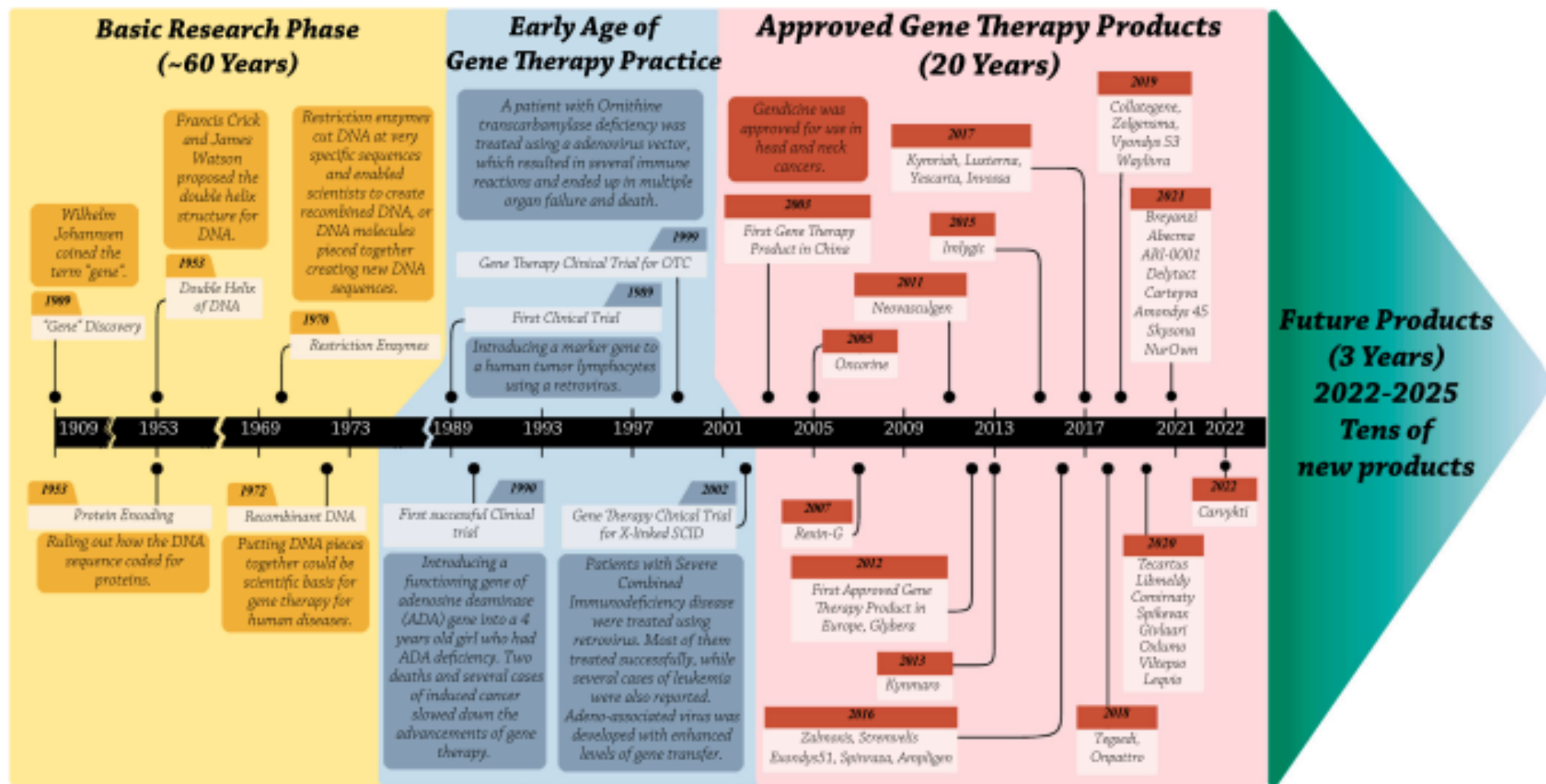
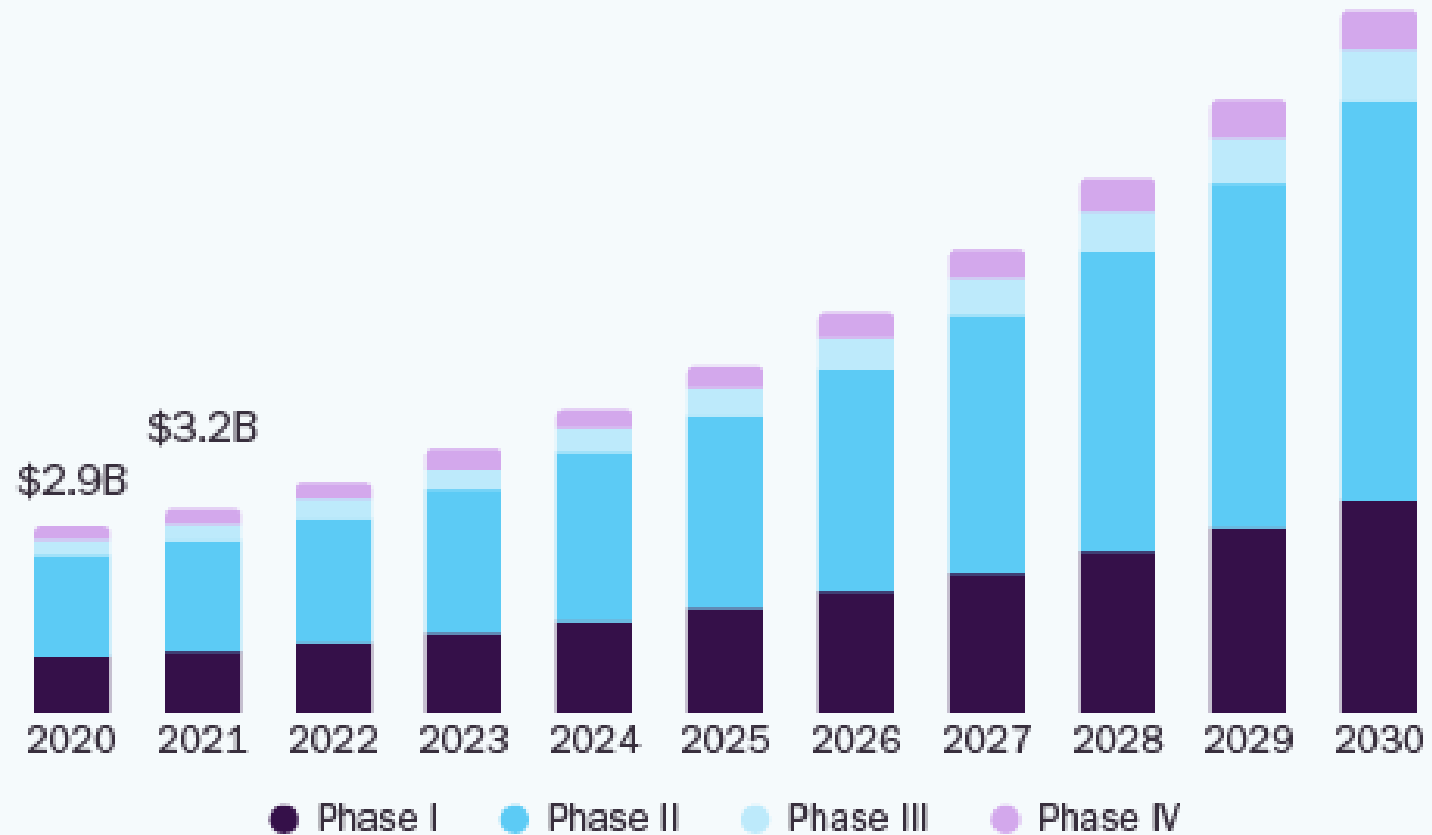


Fig. 2. Historical timeline of gene therapy.

# U.S. Cell & Gene Therapy Clinical Trials Market

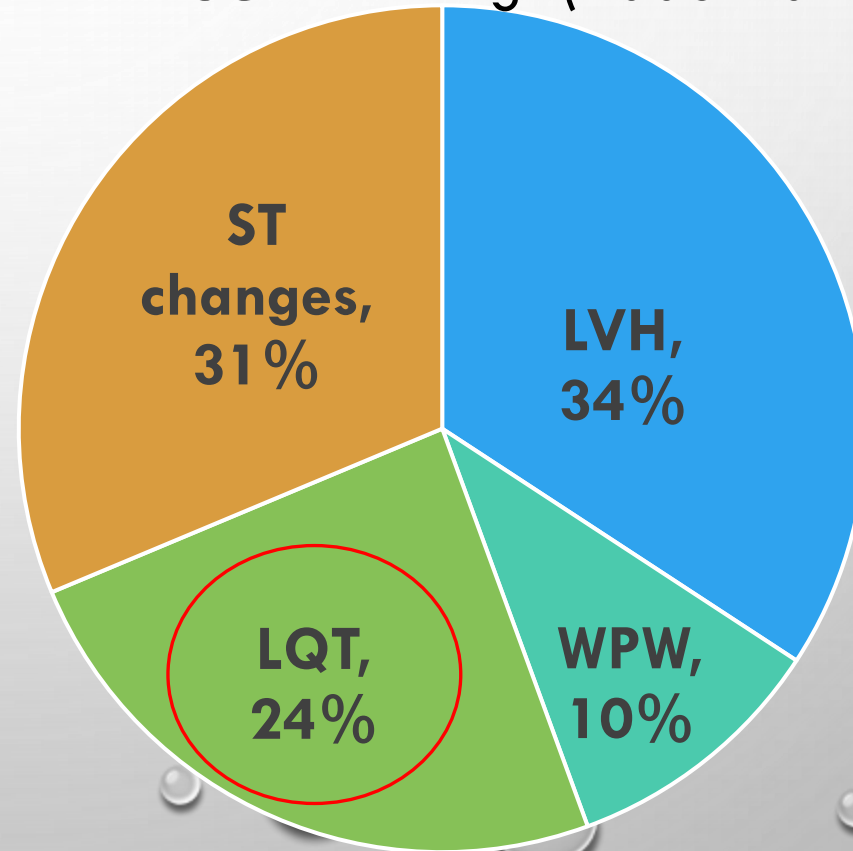
Size, by Phase, 2020 - 2030 (USD Billion)



# CONDITIONS WE IDENTIFY WITH ECG SCREENING

About 1-2% of all ECGs we do have an abnormal finding which requires further evaluation

Summary of abnormal findings from ECG screenings (2006-2011)



The image features a light gray background with a subtle, faint watermark of a globe in the center. The globe shows continents and is surrounded by a circular border. In the corners of the image, there are several realistic water droplets of various sizes, some overlapping, with highlights and shadows that give them a three-dimensional appearance. The text 'THANK YOU' is centered in a bold, black, sans-serif font.

**THANK YOU**