Cardiac Amyloidosis 2025

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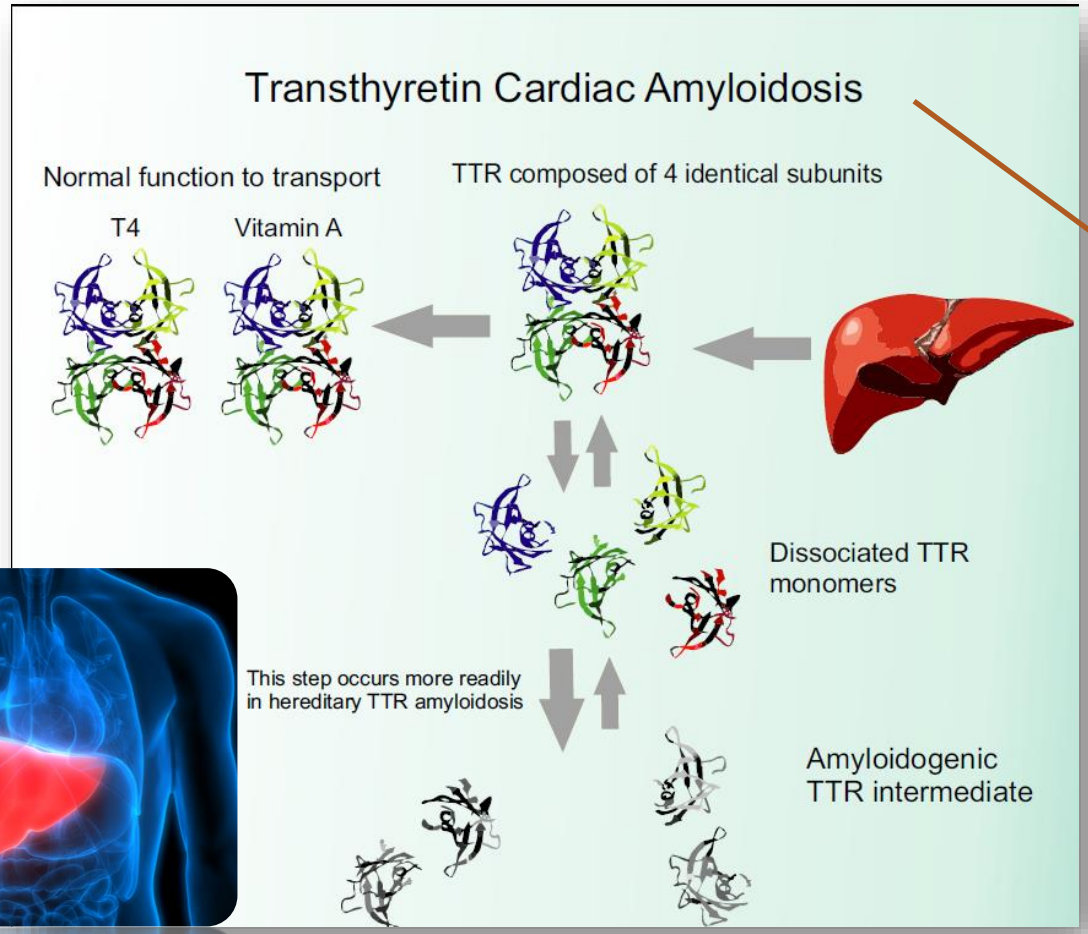
ECHO AUSTRALIA

17-19 March 2025

Amyloid – 2 Diseases

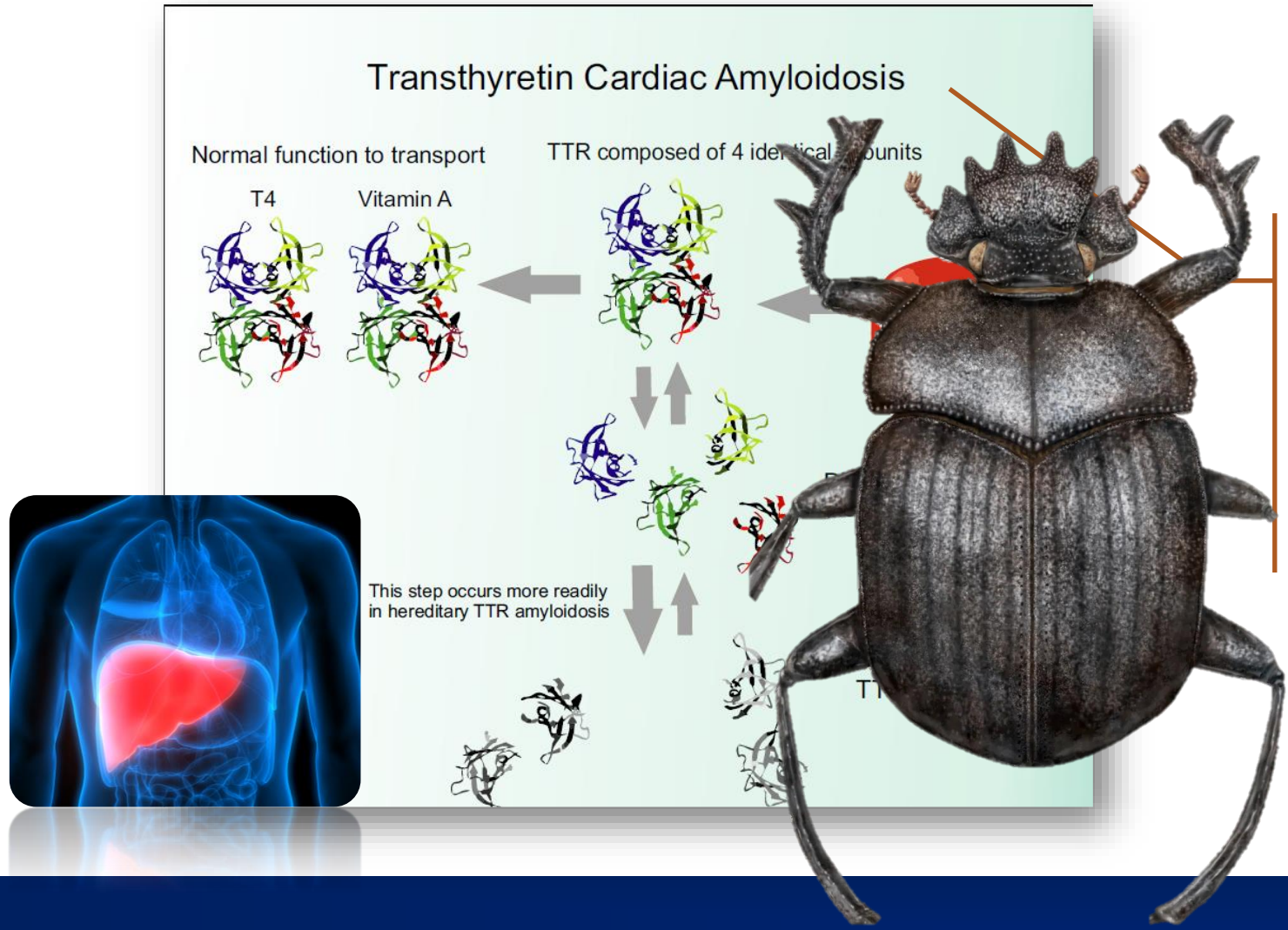


TRANSTHYRETIN (TTR)

TRANSport THYroxine RETINoic acid

From Patel and Hawkins J. Internal Med. 2015; 278: 126-144

Amyloid – 2 Diseases



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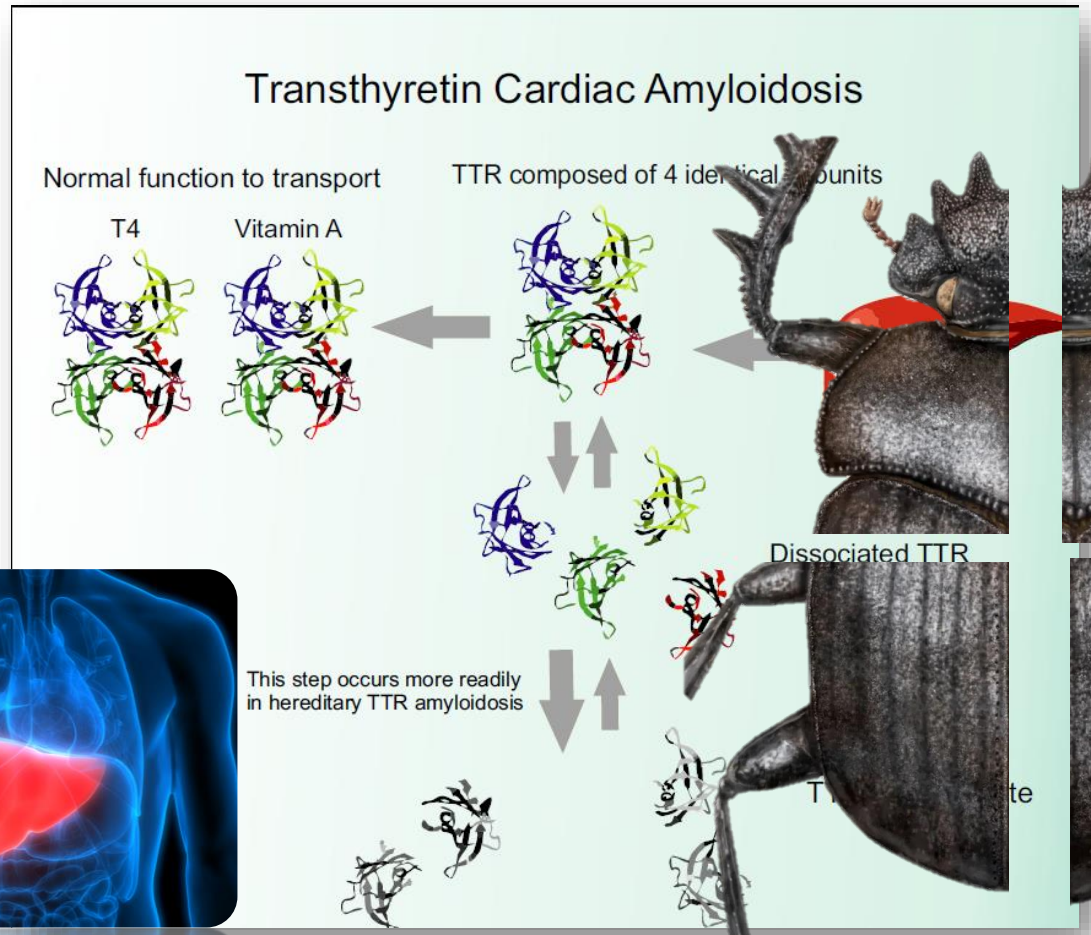

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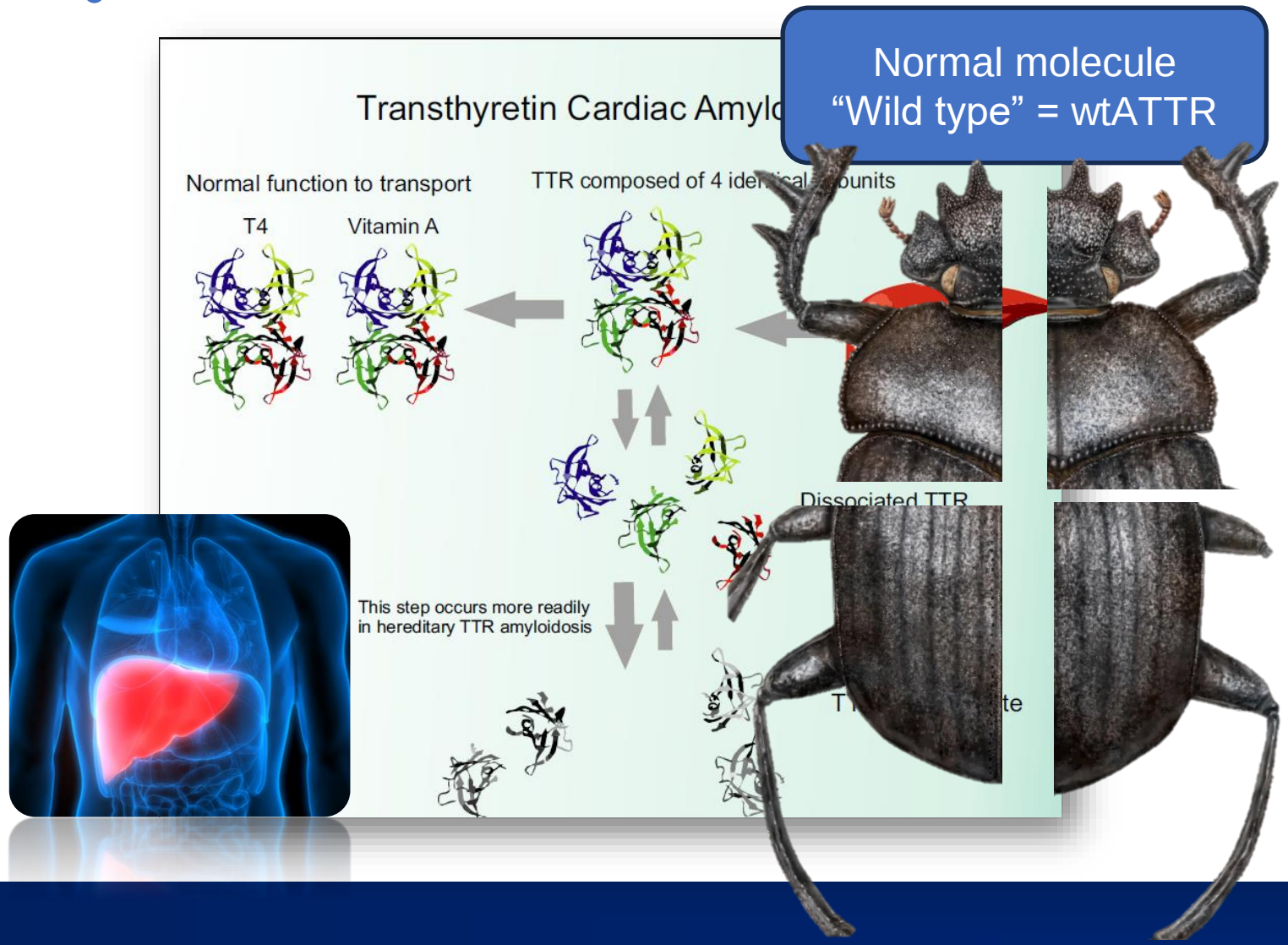
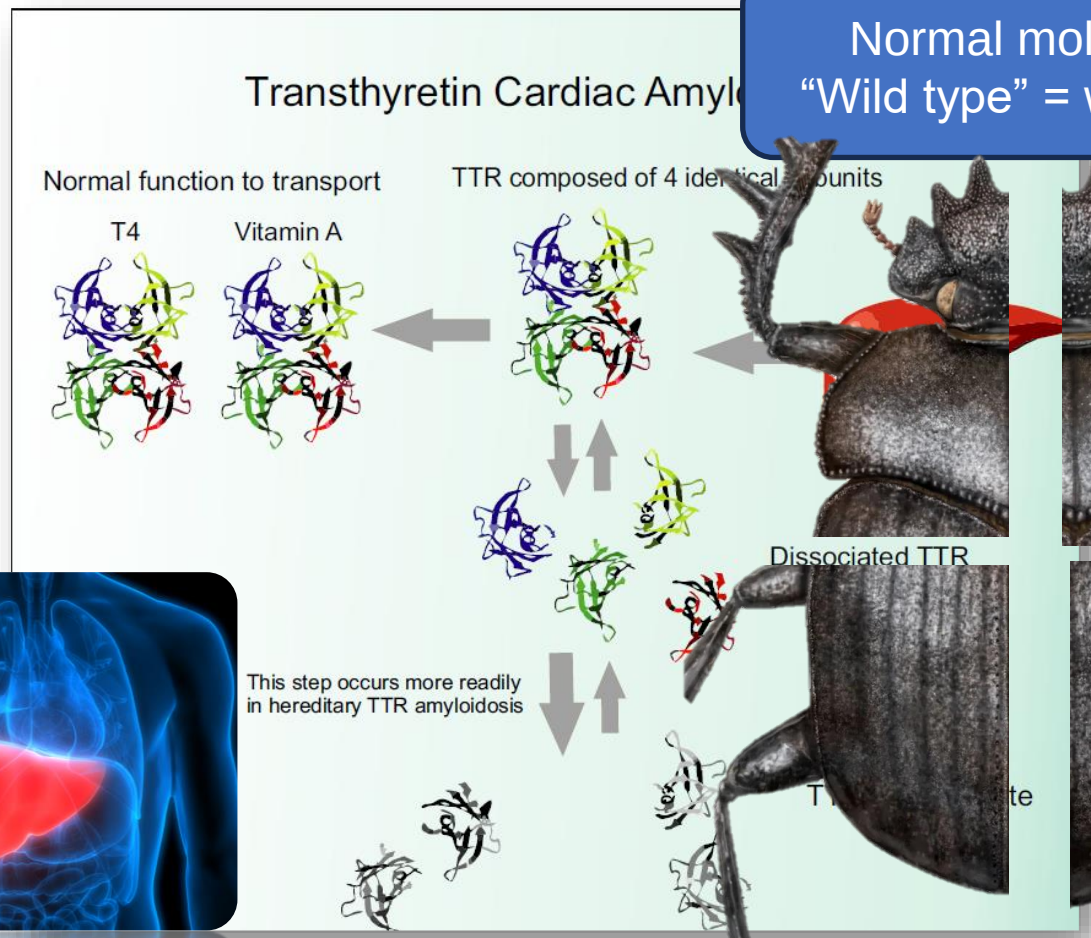


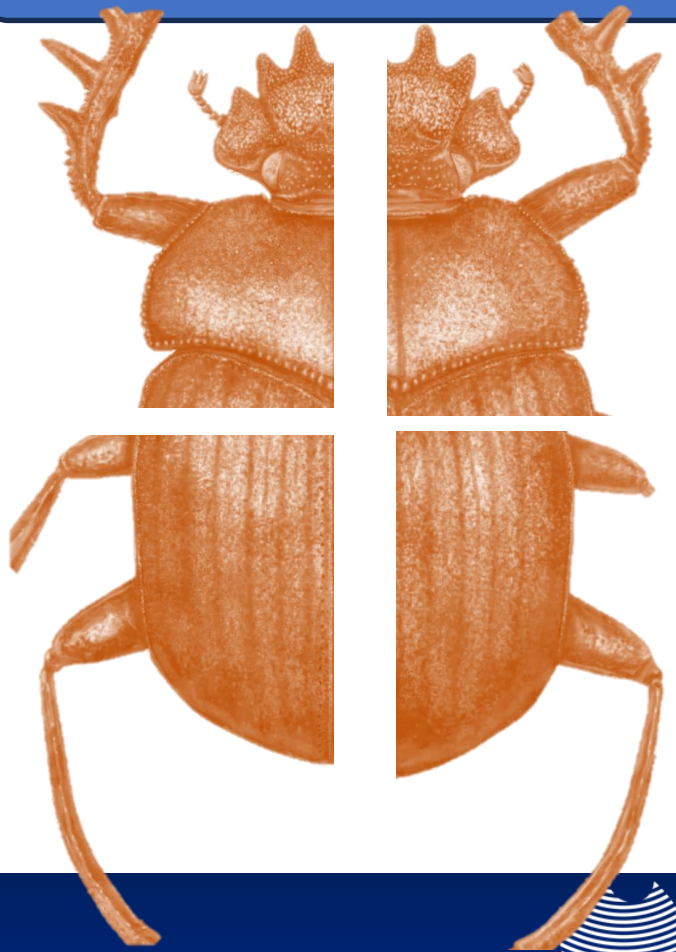
Normal molecule
"Wild type" = wtATTR

Amyloid – 2 Diseases



Normal molecule
"Wild type" = wtATTR

Hereditary/Mutant molecule
= mATTR = hATTR = vATTR



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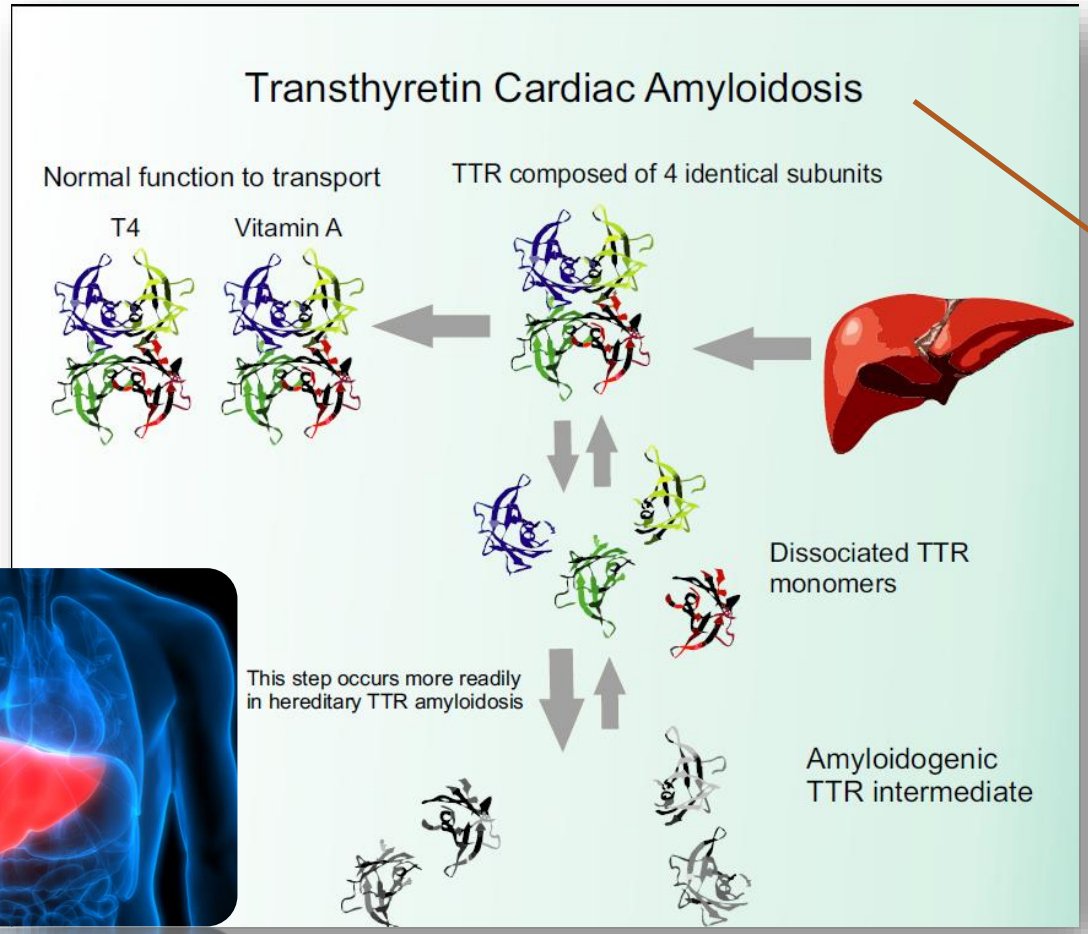
Amyloid – 2 Diseases



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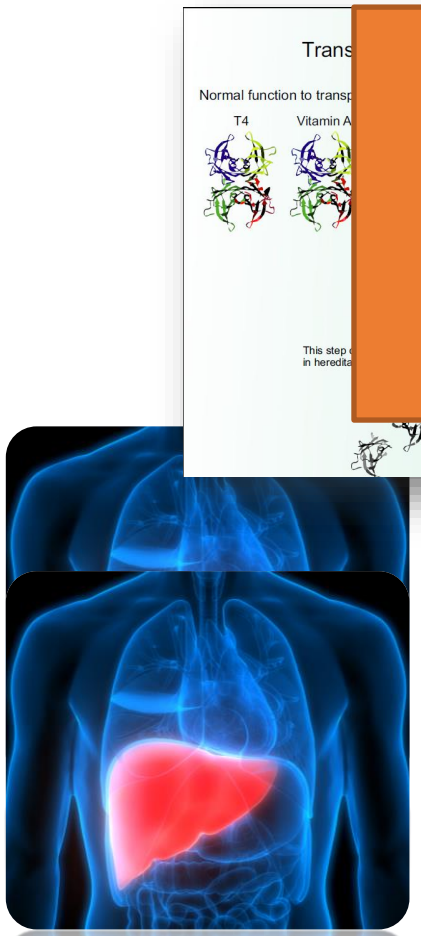
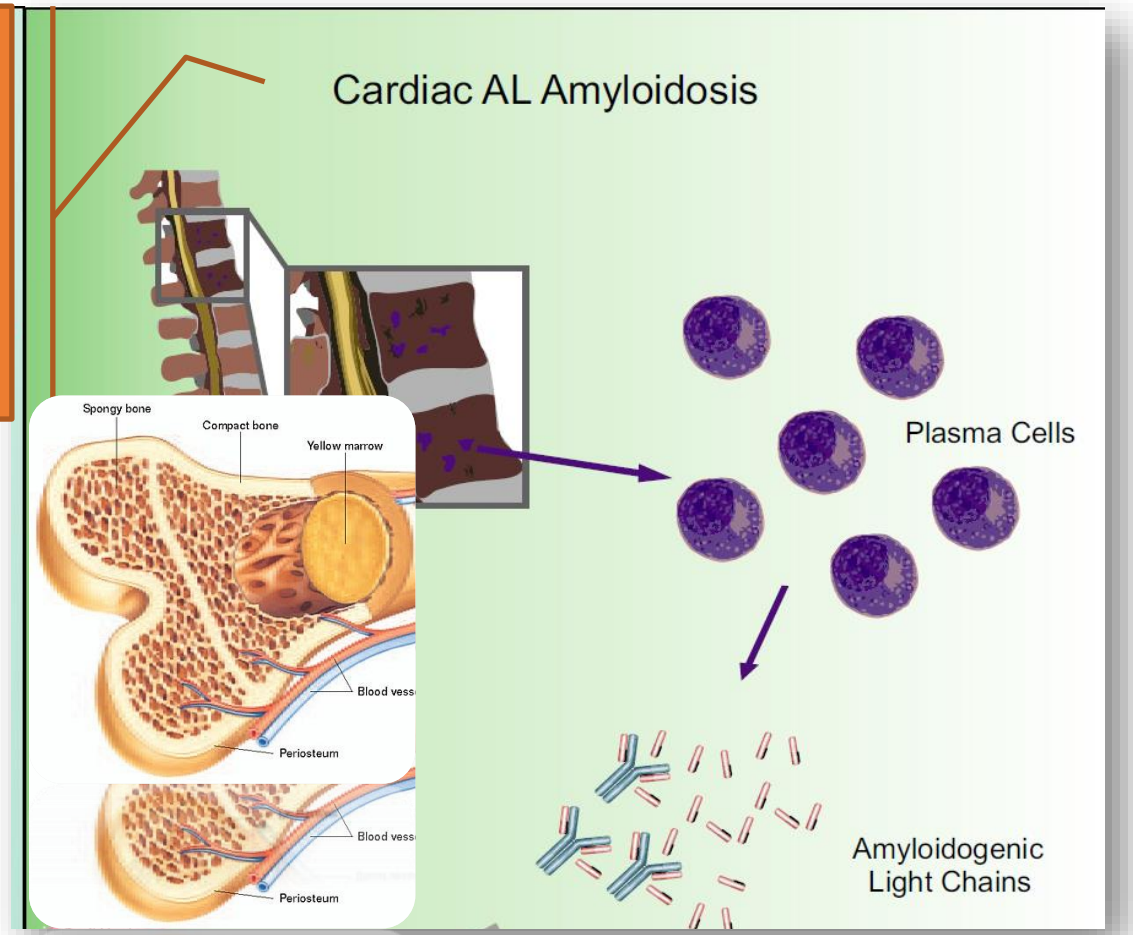
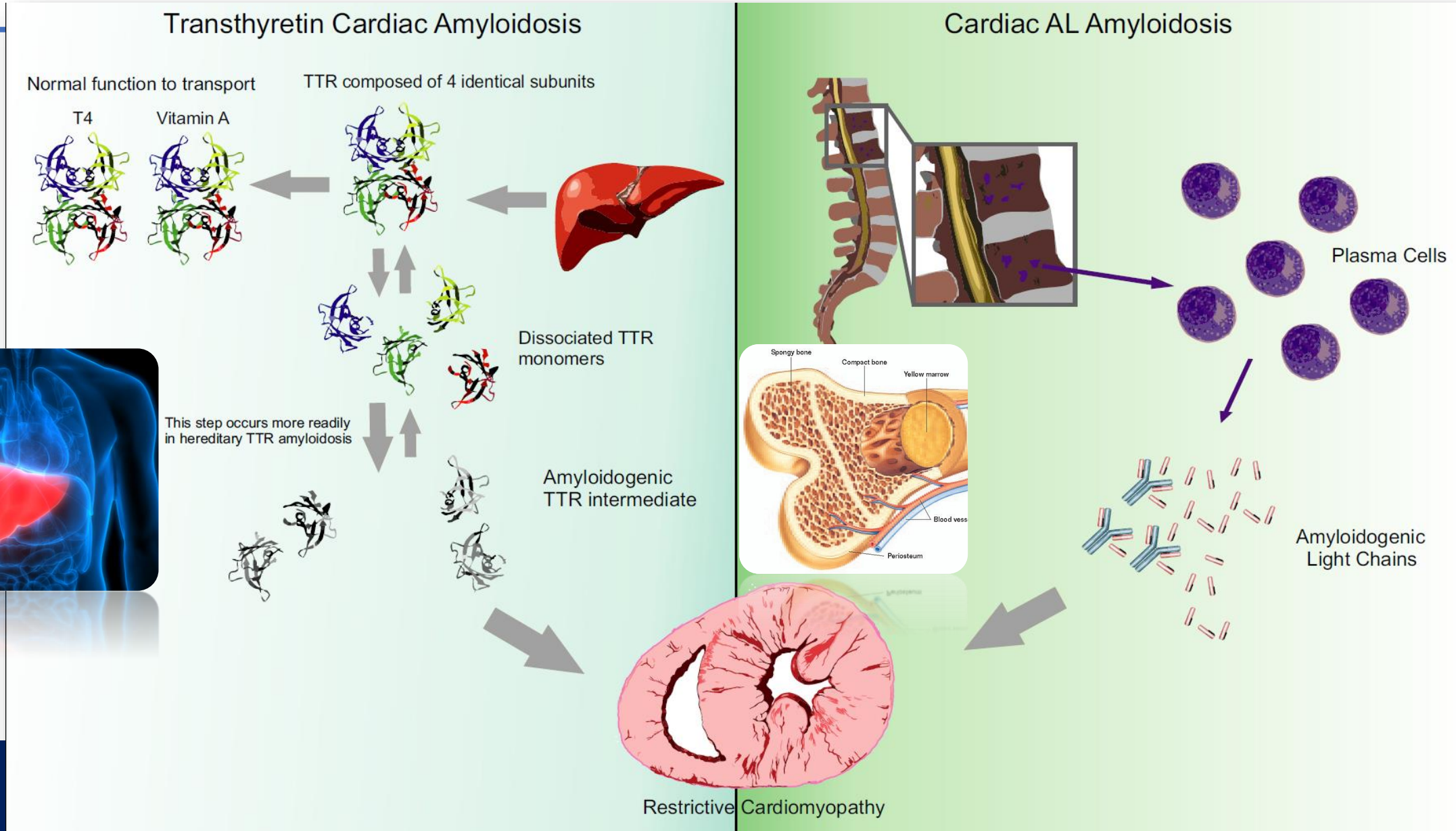


AL Amyloid

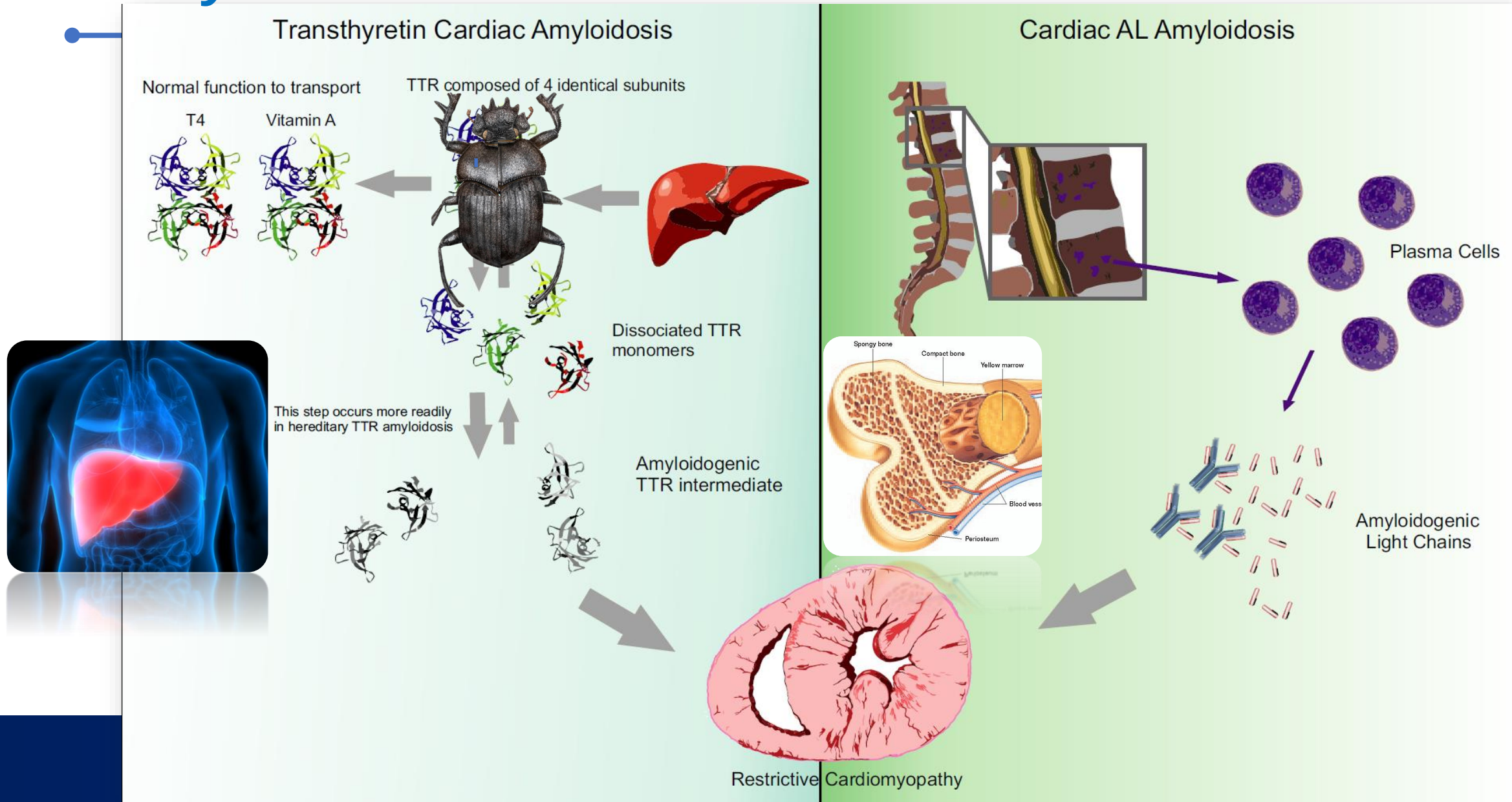
Amyloid Light Chain
Immunoglobulin fragments



Amyloid – 2 Diseases



Amyloid – 2 Diseases



Amyloid – 2 Diseases

Transthyretin Cardiac Amyloidosis

Normal function to transport

T4

Vitamin A

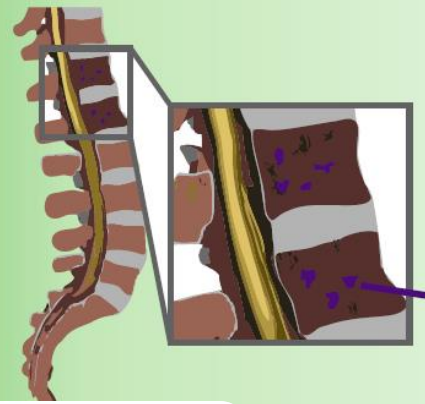
TTR composed of 4 identical subunits

Dissociated TTR monomers

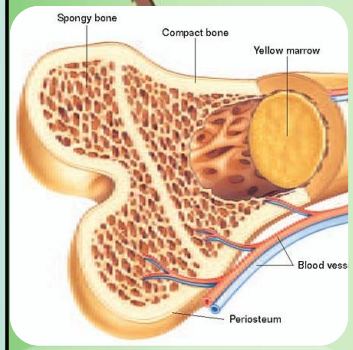
This step occurs more readily in hereditary TTR amyloidosis

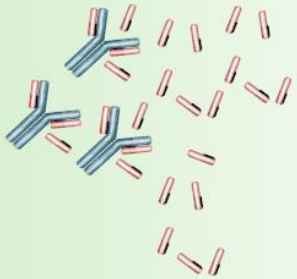
Amyloidogenic TTR intermediate

Cardiac AL Amyloidosis

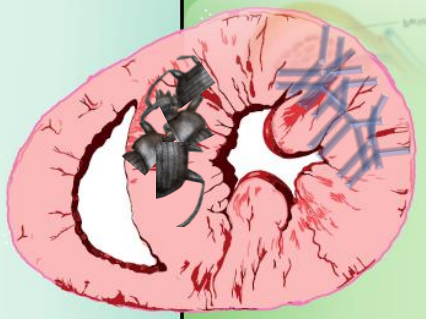


Plasma Cells



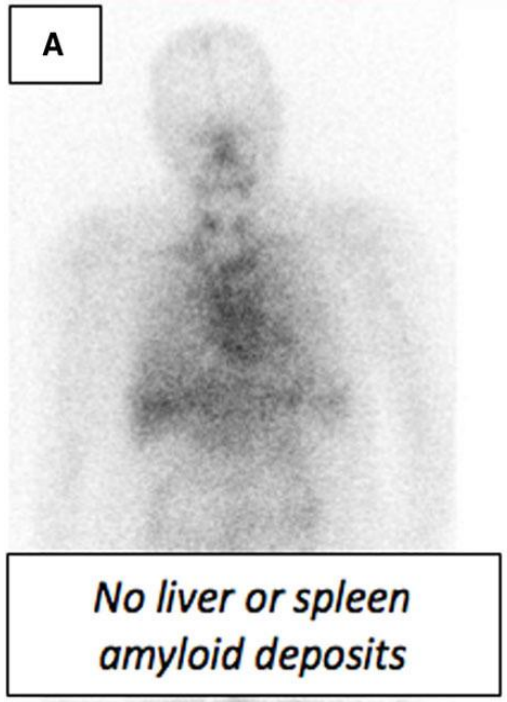


Amyloidogenic Light Chains

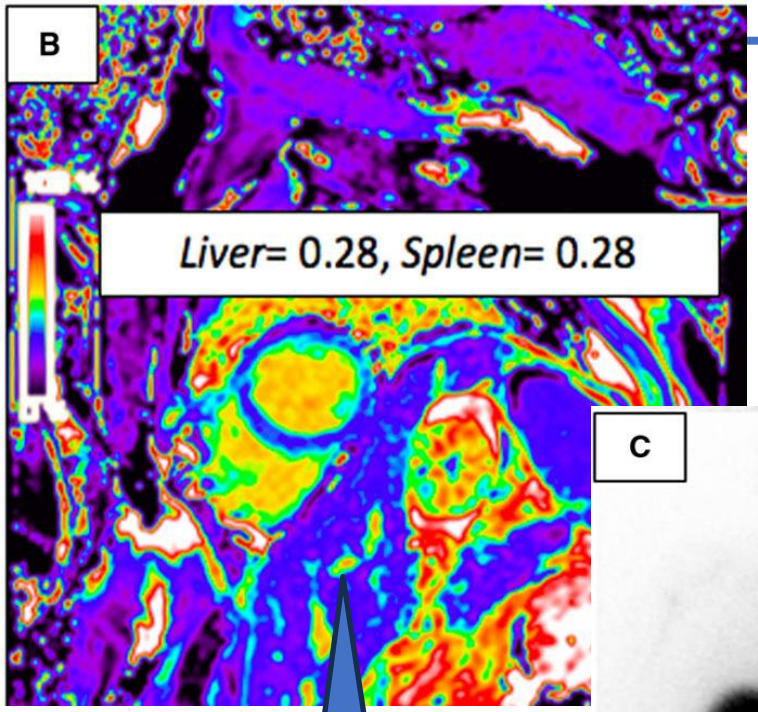


Restrictive Cardiomyopathy

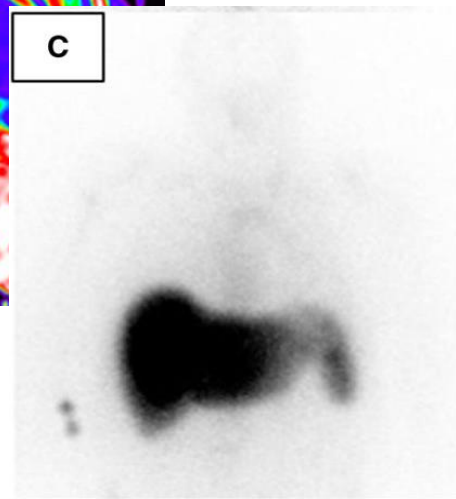
Radiolabelled Serum Amyloid P component



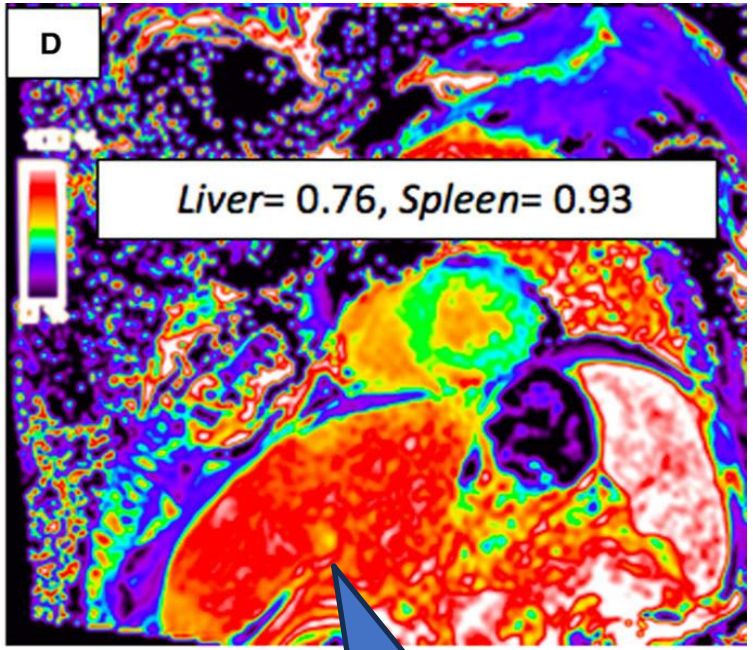
No liver or spleen amyloid deposits



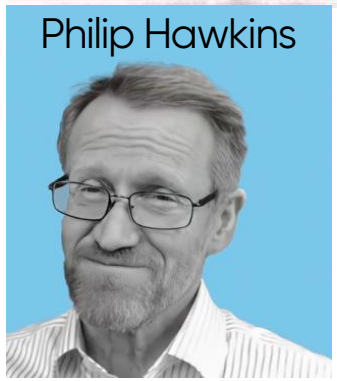
Blue liver
No amyloid



Large liver and spleen deposits

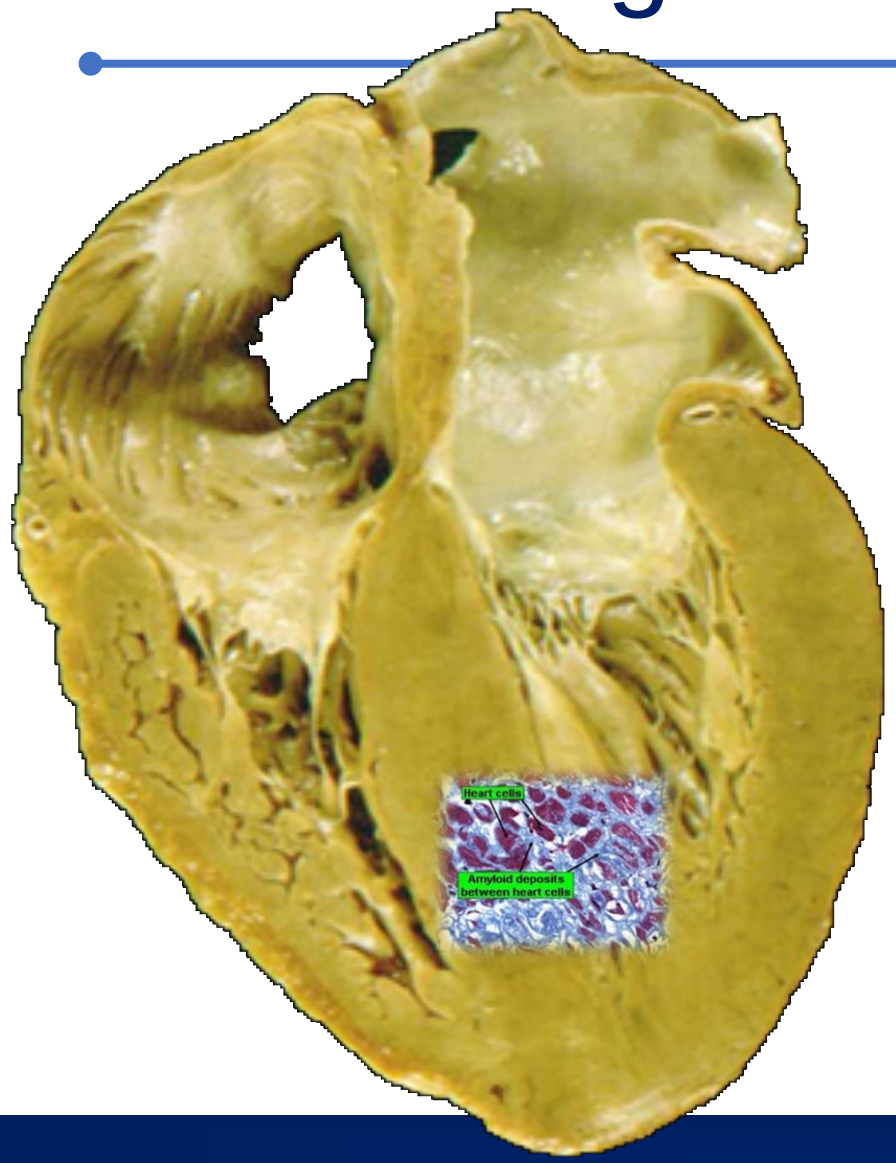


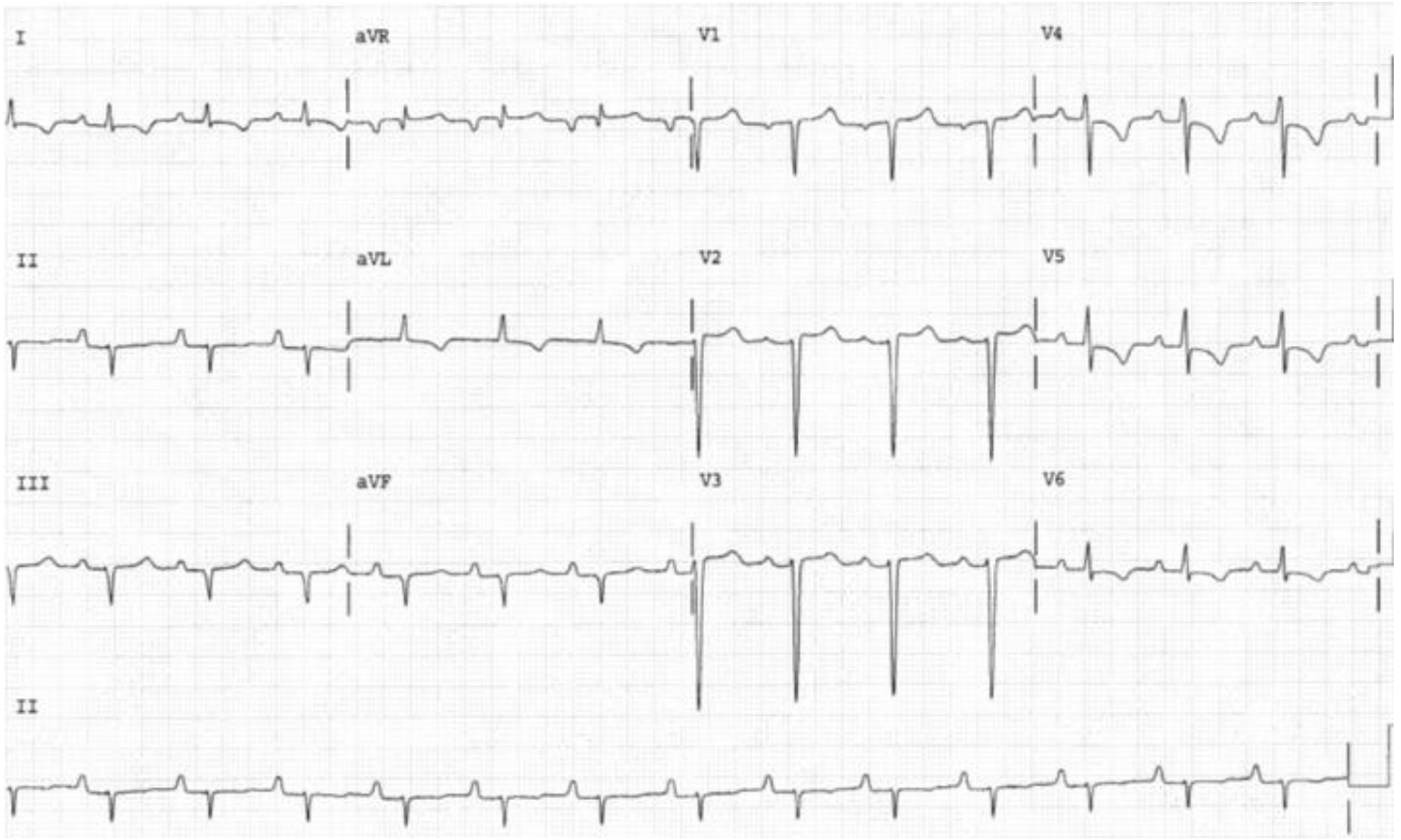
Red liver
Amyloid+++



Liza Chacko. Circulation: Cardiovascular Imaging. Cardiac Magnetic Resonance–Derived Extracellular Volume Mapping for the Quantification of Hepatic and Splenic Amyloid, Volume: 14, Issue: 4, DOI: (10.1161/CIRCIMAGING.121.012506)

Low voltage ECG





ECG changes in different amyloid

Circulation: Heart Failure
Volume 13, Issue 3, March 2020
https://doi.org/10.1161/CIRCHEARTFAILURE.119.006619

RESEARCH LETTER

Baseline ECG Features and Arrhythmic Profile in Transthyretin Versus Light Chain Cardiac Amyloidosis

Francesco Cappelli, MD*, Elisa Vignini, MD*, Raffaele Martone, MD, Stefano Perlini, MD, PhD, Roberta Mussinelli, MD, Anna Sabena, MD, Sofia Morini, MD, Martina Gabriele, MD, Giulia Taborchi, MD, Simone Bartolini, MD, Angelica Lossi, MD, Giulia Nardi, MD, Niccolò Marchionni, MD, PhD, Carlo Di Mario, MD, PhD, Iacopo Olivotto, MD, and Federico Perfetto, MD, PhD

- 40% wtATTR patients presented with AF compared with 20% of mTTR and only 6% of those with AL
- AV block, and intraventricular delay were also more common among ATTR patients, leading to a significant prevalence of device implantation before diagnosis.
- AL subjects more often presented in sinus rhythm and displayed the typical low voltage pattern at diagnosis.

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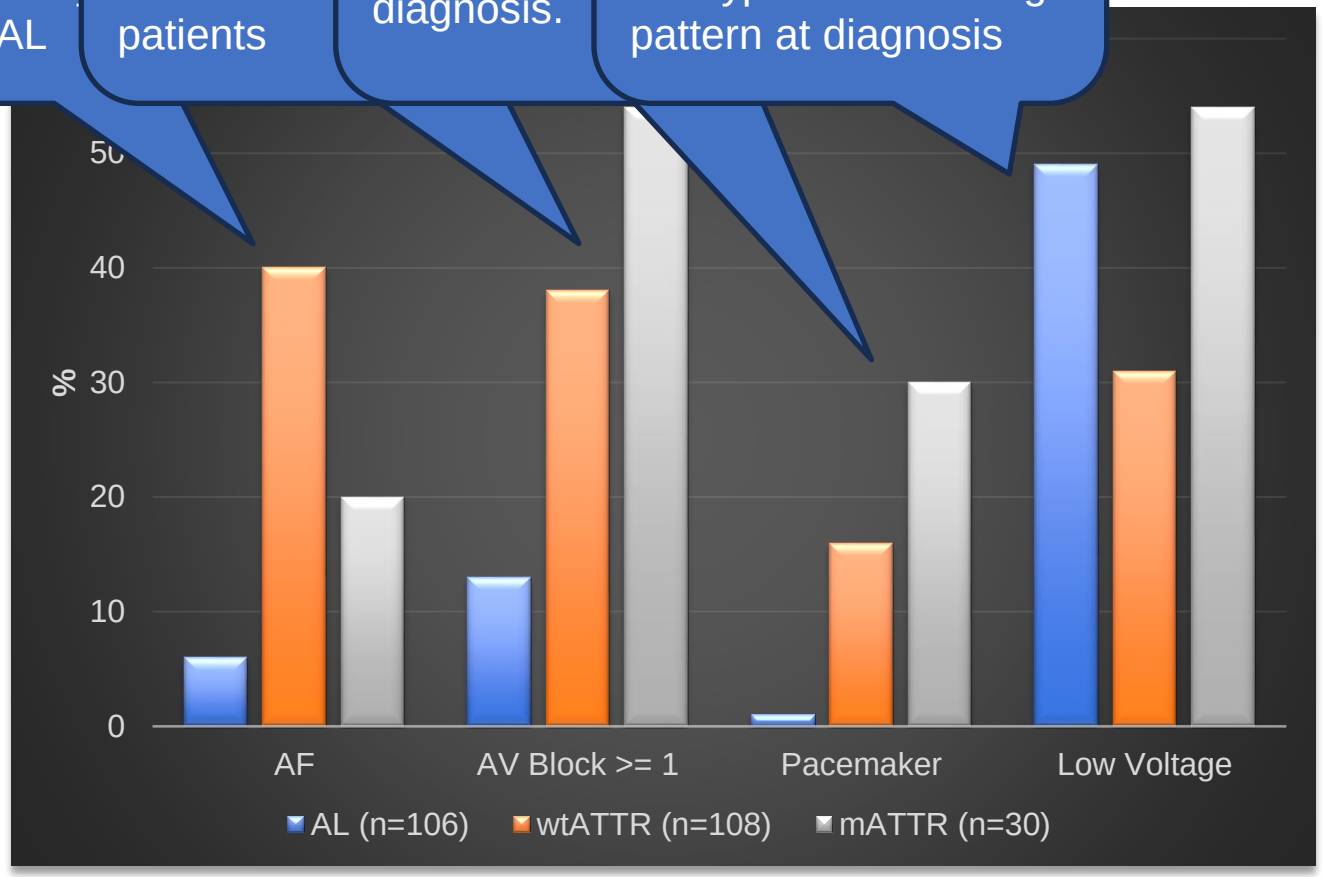
AL subjects more often presented in sinus rhythm and displayed the typical low voltage pattern at diagnosis



ECHO AUSTRALIA

Echo

ASE American Society of Echocardiography — Sound Saves Lives

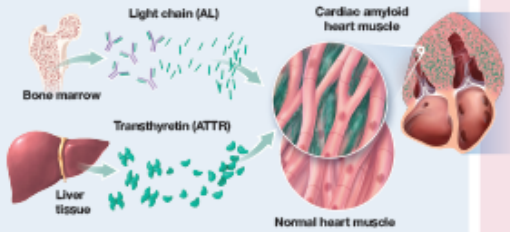
Echo for Cardiac Amyloidosis

Pfizer

What is Cardiac Amyloidosis (CA)?

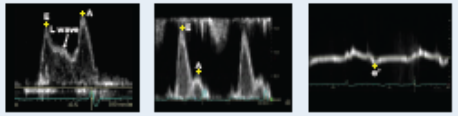
A form of restrictive infiltrative cardiomyopathy due to deposition of amyloid fibrils in the myocardium.

There are 2 common types. Similar echo features are seen in both types of CA.



Doppler Echocardiography in CA

Progressive diastolic dysfunction is a feature of CA.
This may only be mildly abnormal in early stages of the disease.



Pulsed wave Doppler of the mitral inflow:

This ranges from a low E/A ratio (<0.8) suggesting restrictive hemodynamics. Abnormal relaxation pattern is less common in CA but may be present in early stage. Note L wave is a clue to elevated filling pressure.

Tissue Doppler:

Strongly suggestive:
- Mitral annular TDI < 5 cm/sec
- Small A wave in sinus rhythm

Not suggestive:
- Septal or lateral tissue Doppler e' > 10 cm/s

Rule of 5 (5-5-5)

All e', a', s' velocities < 5 cm/s

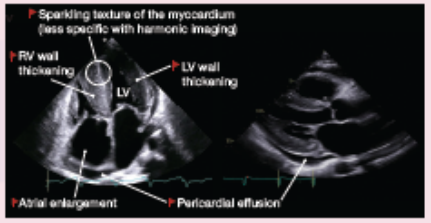
This is a clue to the diagnosis of CA.

Key Points

- Echo may be the first clue to the diagnosis of amyloidosis.
- Classic: thickened myocardium, diastolic dysfunction, and abnormal strain (apical sparing)
- Atypical or subtle findings may be seen in early disease
- Consider strain imaging whenever amyloid suspected
- Echo alone is not diagnostic of CA, nor can it differentiate between AL and ATTR.

Echo Red Flags

Apical 4 chamber view (left) and parasternal long axis view (right) displaying the typical features of CA.



- Reduced mitral TDI velocities
- Reduced GLS with apical sparing
- Low flow low gradient AS
- Diastolic dysfunction (≥grade 2)

Clinical Red Flags

- Heart failure
- Nephrotic syndrome
- Peripheral or autonomic neuropathy
- Weight loss
- Bilateral carpal tunnel
- Spinal stenosis
- Periorbital purpura

Strain Analysis in CA

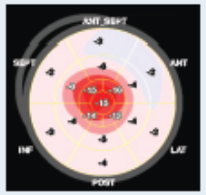
Myocardial deformation ("strain") measured by 2-dimensional speckle tracking imaging is very useful in CA. Longitudinal strain is the measure of the longitudinal contractile function of the heart.

When to do strain:

If feasible anytime there is increased LV wall thickness, especially in:
- Over 65 year olds
- Heart failure
- No history of poorly controlled HTN

Global longitudinal strain (GLS):

Normal values of GLS vary between vendors; normal is usually considered to be more negative than -20% with an SD of ~ 2% (lower limit of normal -16% to -18%, depending on vendor).
Values nearing 0% suggest more dysfunction and either advanced disease or disease progression.



Strain pattern:

In cardiac amyloidosis the segmental strain curves representing the apical segments will have a further deflection away from the 0 line than the curves representing the basal segments. When plotted on a bullseye, this will generate a characteristic "apical sparing" pattern visually.

Strain ratios:

Longitudinal strain ratios that have been described, with the diagnostic cutoffs used in the original publications: proposed ratios incorporating LV GLS for diagnosis of CA.

Sara A.M. Cuddy, MD; Michael Chetrit, MD; Madeline Jankowski, BS, RDCS, ACS, FASE; Milind Desai, MD, MBA; Rodney H. Falk, MD; Rory B. Weiner, MD, FASE; Allan L. Klein, MD, FASE; Dermot Phelan, MD, PhD, FASE; Martha Grogan, MD. Practical Points for Echocardiography in Cardiac Amyloidosis. JASE 2022; 35(5): A31-A40.

PP-VDM-USA-1525

Poster ordering information available at: ASEcho.org

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Access resources on Amyloidosis and full Journal article:

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Echo for Cardiac Amyloidosis

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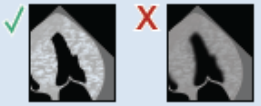
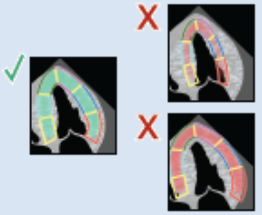
Tips for Performing Strain Analysis

Increased gain:
Higher gain results in more speckles; need good endocardial definition.



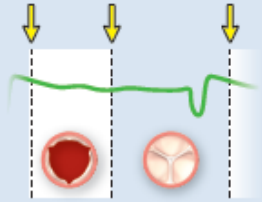
Region of interest (ROI):
Include 90% of the myocardium as error can occur if too narrow, favoring epicardial or endocardial regions. If too wide, it can lead to abnormal, often lower, values.



Consistent sector width and depth:
Wide enough to include the full wall thickness and apex and extend beyond annulus to allow capture of entire LV throughout cardiac cycle.

Longitudinal follow-up:
Using a consistent vendor allows for better comparisons over time.

ECG gating and timing of end diastole and end systole to aortic valve opening and closing:
It is very important for evaluation of end-systolic versus peak strain values; deformation after aortic valve closure is not relevant.
If ECG gating is incorrect, e.g., tracking p wave, may need to manually adjust off-line.



Standardized Acquisition and Interpretation of Echocardiography for CA

(Adapted from Expert Consensus Recommendations*)

Parameter for acquisition and reporting	Abnormal parameter	Notes
LV wall thickness	Increased LV wall thickness (>1.2 cm) and increased relative wall thickness (>0.42)	Discordance between increased LV wall thickness relative to ECG QRS voltage is particularly suggestive, but normal ECG voltage can also be seen.
Myocardial echogenicity	Increased echogenicity of the myocardium (sparkling, hyperrefractile "texture" of the myocardium)	Not highly specific (differential diagnosis includes endstage renal disease or other infiltrative cardiomyopathies); highly suggestive in conjunction with severely reduced longitudinal function of the LV.
Atrial size and function	Atrial enlargement and dysfunction (see diastolic function)	Nonspecific but important finding to support the diagnosis and potentially provide insight into risk for stroke or arterial embolism.
Interatrial septum and valves	Increased thickening of the interatrial septum and valves (>0.5 cm)	Nonspecific but suggestive of the diagnosis.
Estimated PA systolic and right atrial pressure	Increased pressures (>35 mm Hg for PA, ≥10 mm Hg for right atrium)	These are important parameters to estimate volume status and optimize diuretic dosing.

LS, Longitudinal strain; PA, pulmonary artery.

*Dorbala S, et al. ASNC/AHA/ASE/EANM/HFSA/ISA/SCMR/SNMMI expert consensus recommendations for multimodality imaging in cardiac amyloidosis: part 1 of 2—evidence base and standardized methods of imaging. J Nucl Cardiol 2019;26:2065-123.

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Access resources on Amyloidosis and full Journal article:

ECHO AUSTRALIA

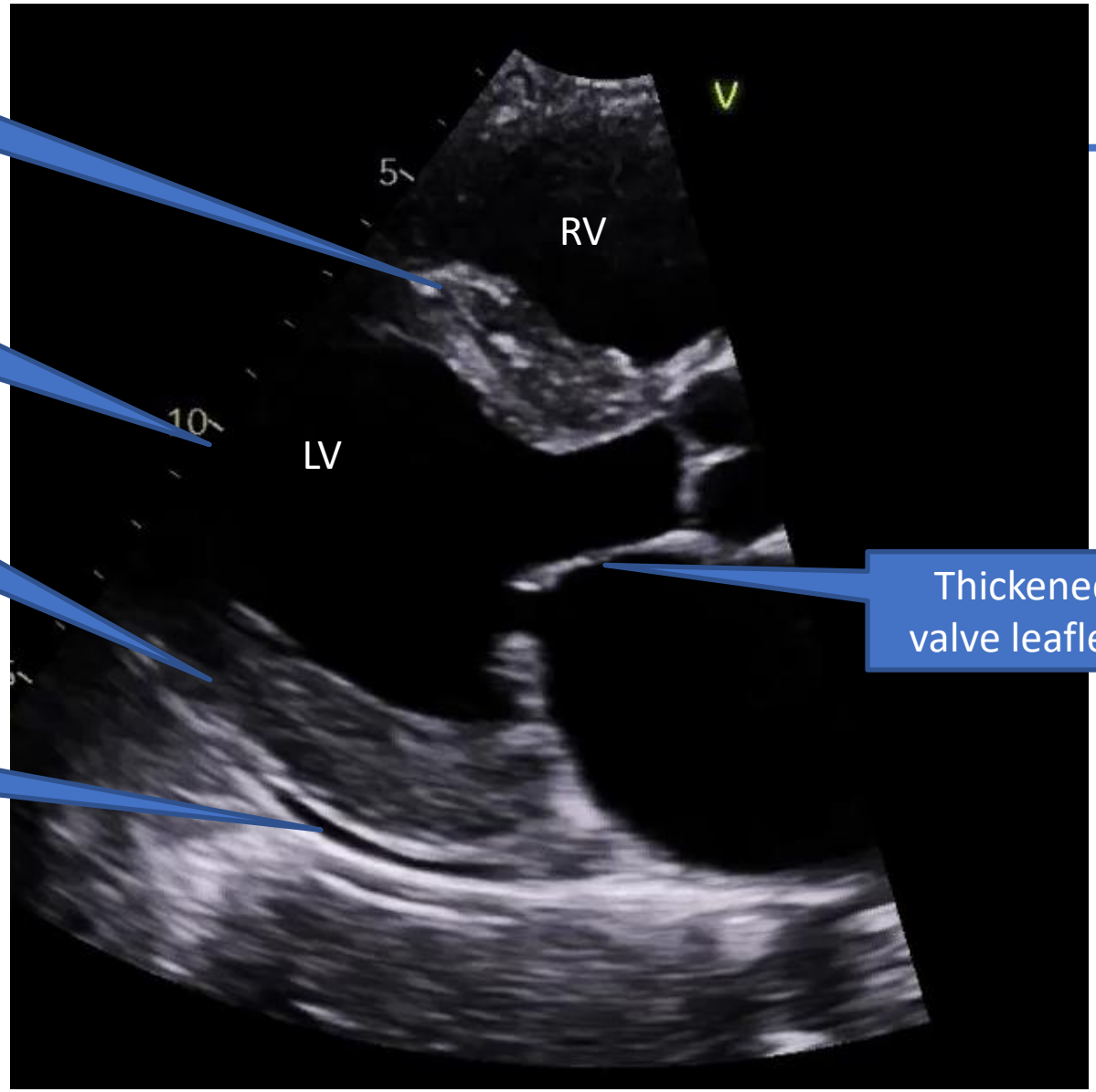
Echo

Thickened "sparkling" Myocardium

Normal LV size and systolic function

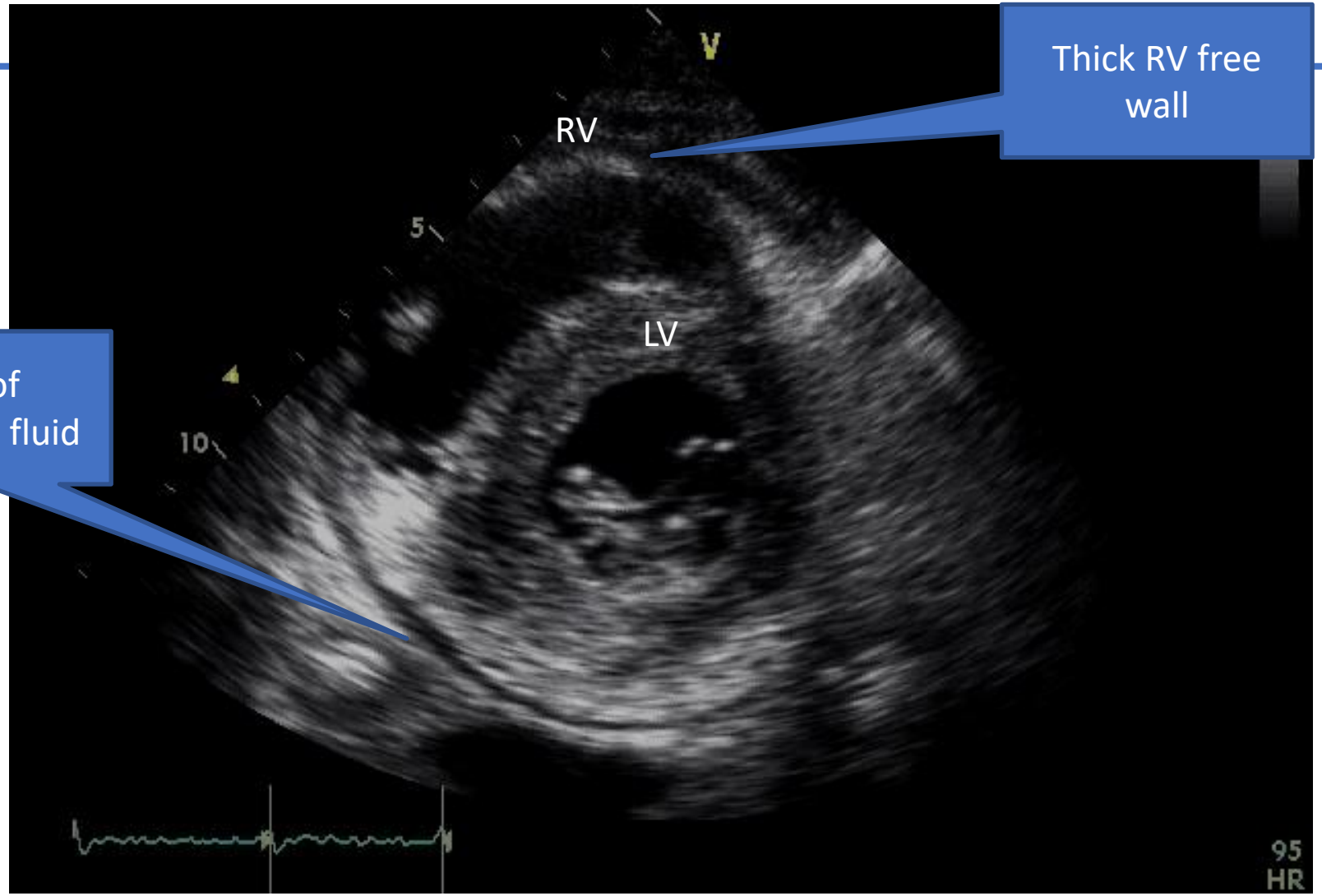
Thickened "sparkling" Myocardium

Sliver of pericardial fluid



Thickened valve leaflets

Echo



Thick RV free wall

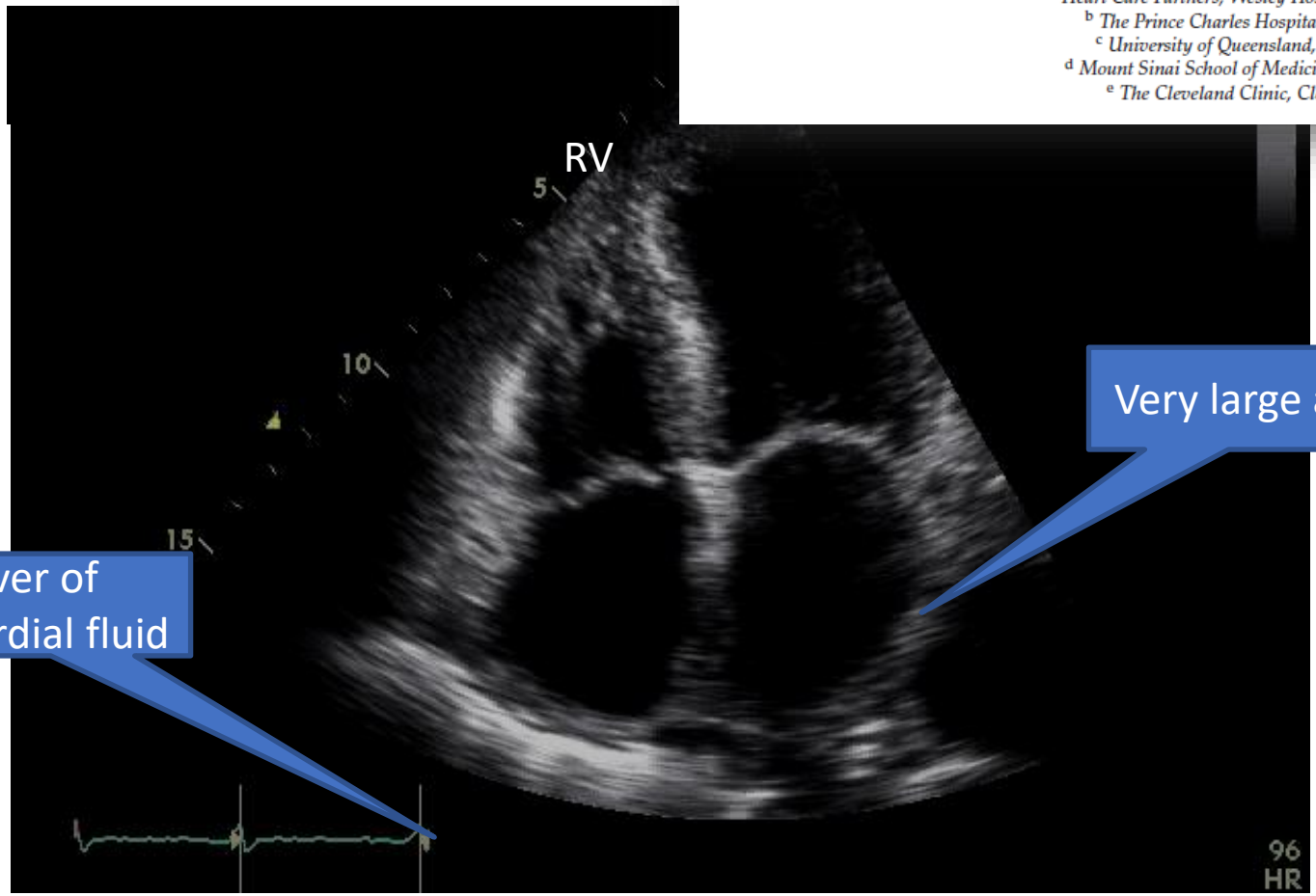
Sliver of pericardial fluid

Amyloid – LA size

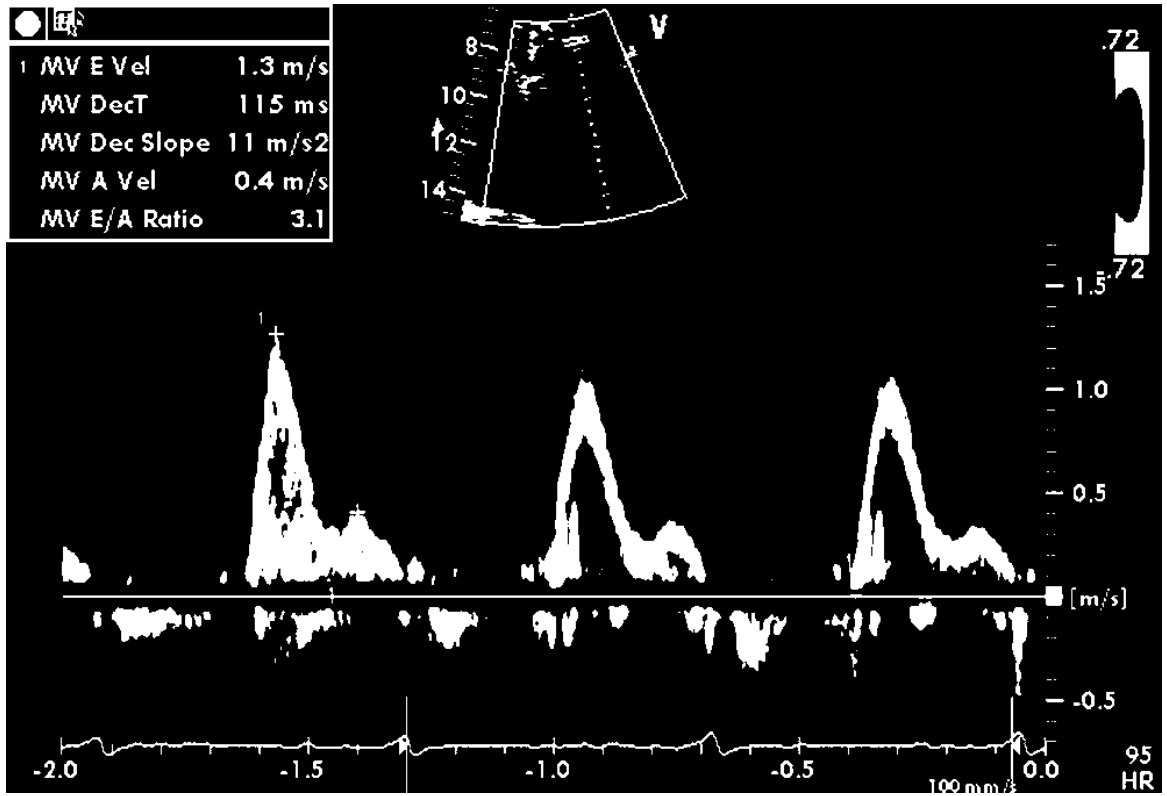
Left atrial size—Another Differentiator for Cardiac Amyloidosis

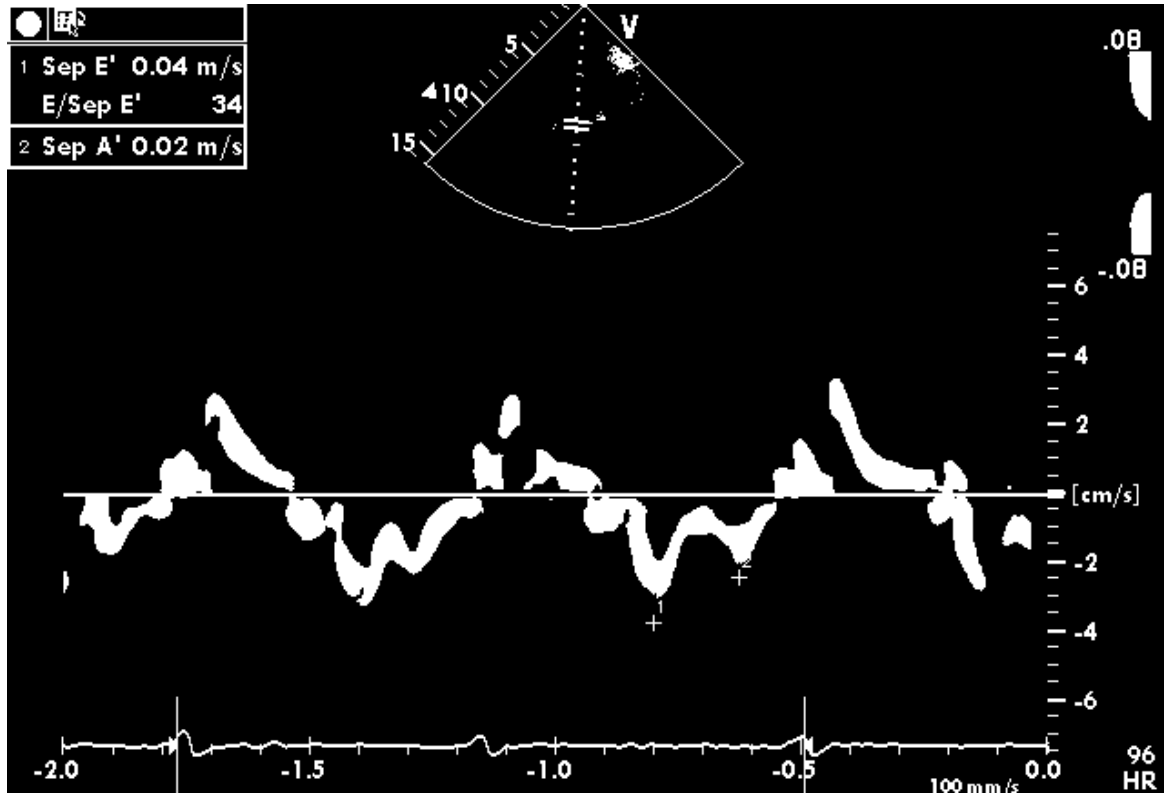
Benjamin T. Fitzgerald, FRACP[a,b,*], Gregory M. Scalia, FRACP[a,b,c], Peter A. Cain, FRACP[a], Mario J. Garcia, FACC[d] and James D. Thomas, FACC[e]

[a] *Heart Care Partners, Wesley Hospital, Brisbane, Australia*
[b] *The Prince Charles Hospital, Brisbane, Australia*
[c] *University of Queensland, Brisbane, Australia*
[d] *Mount Sinai School of Medicine, New York, NY, USA*
[e] *The Cleveland Clinic, Cleveland, OH, USA*



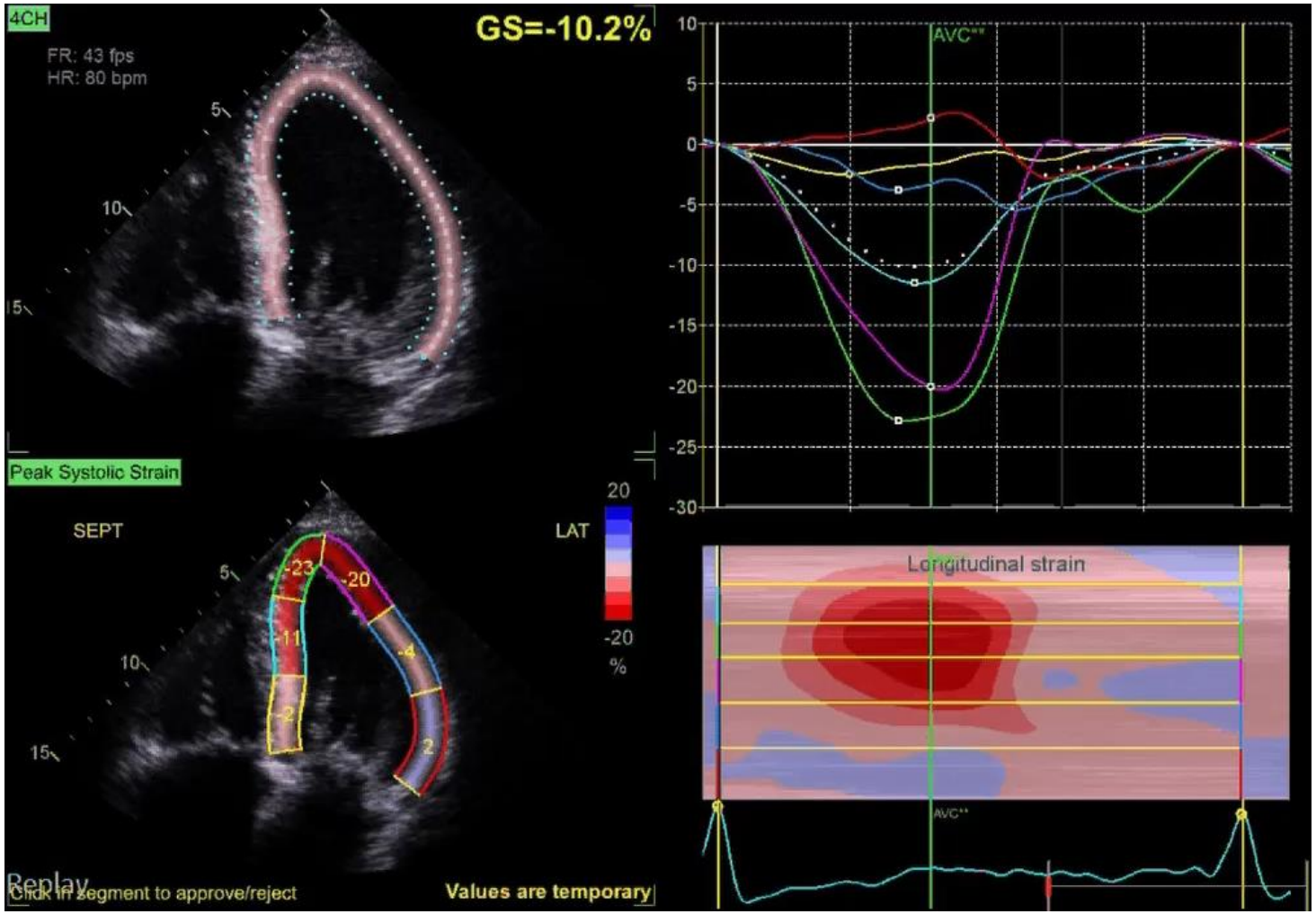
Severe diastolic dysfunction



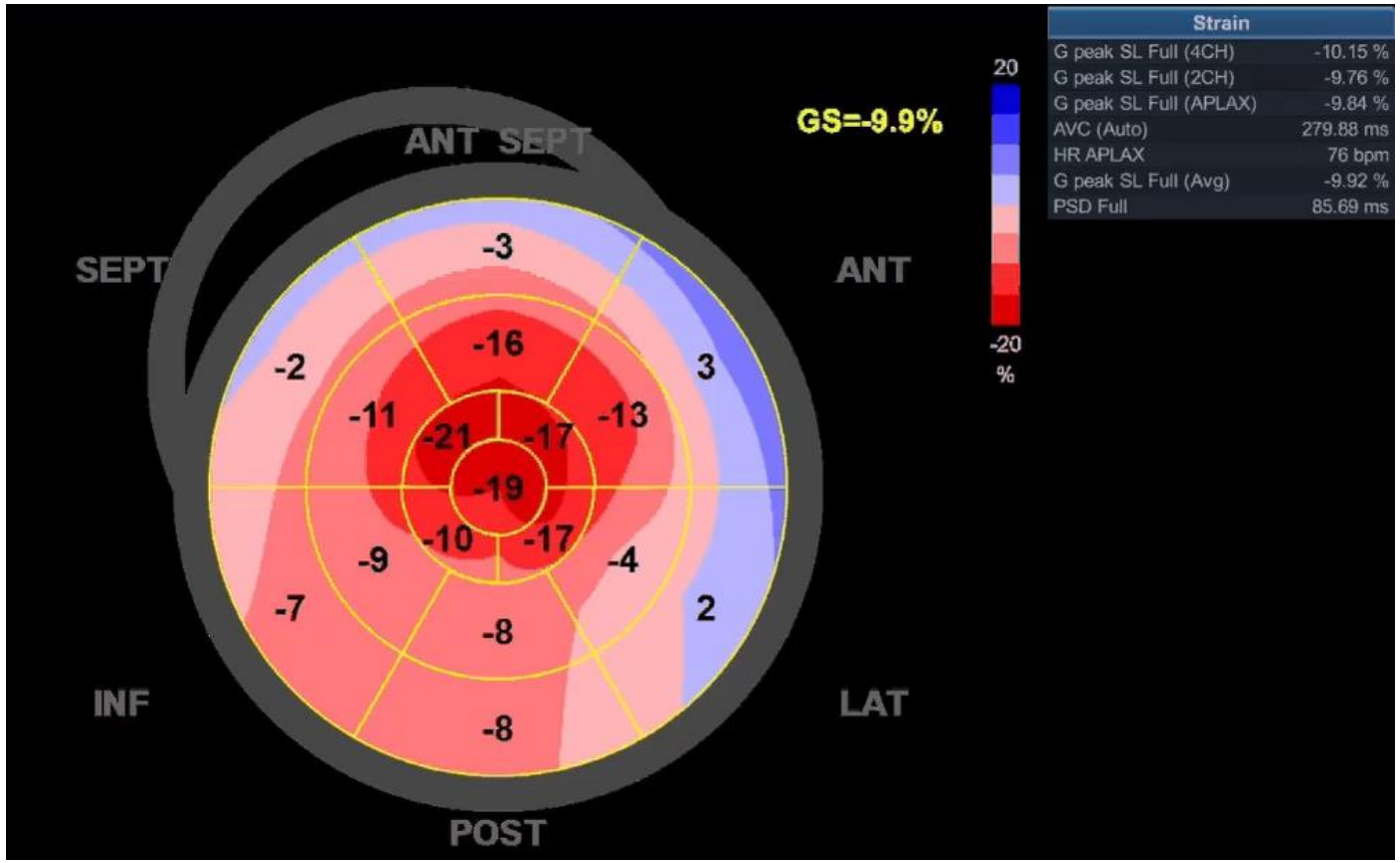


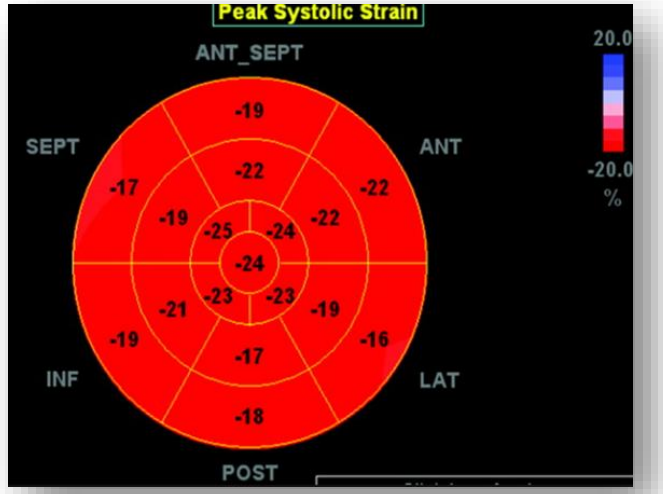
Deceleration time 115ms
E/e' 34
Grade 3 Diastolic dysfunction

Amyloid - Strain Imaging



Amyloid - Strain Imaging





Classical apical sparing Pattern

Averaged GLS = 9.9%

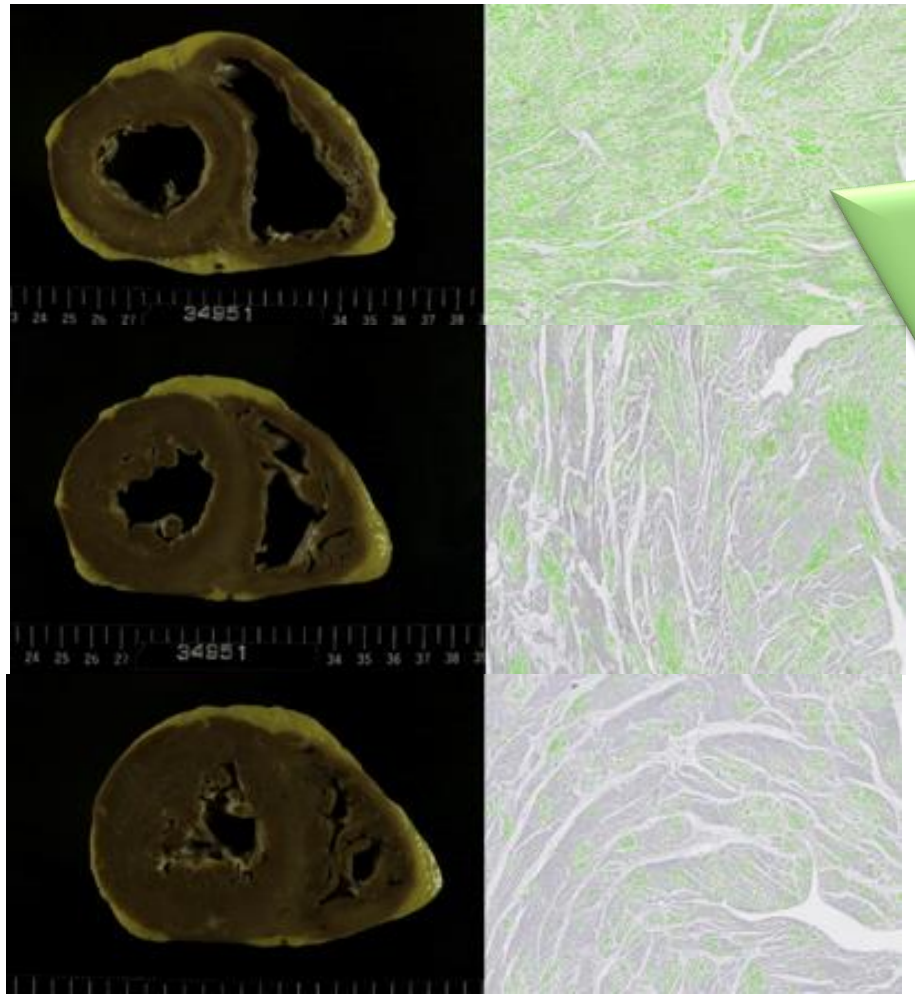
Normal averaged GLS >17%

Amyloid - Strain Imaging

Basal

Mid

Apical

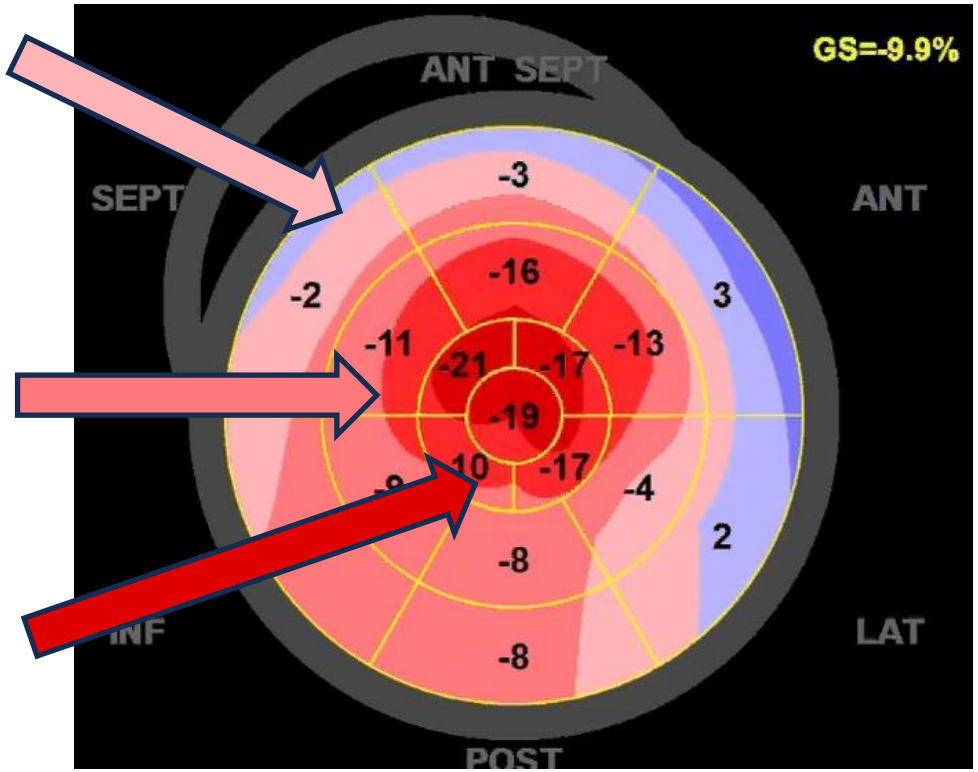


Amyloid Stain %

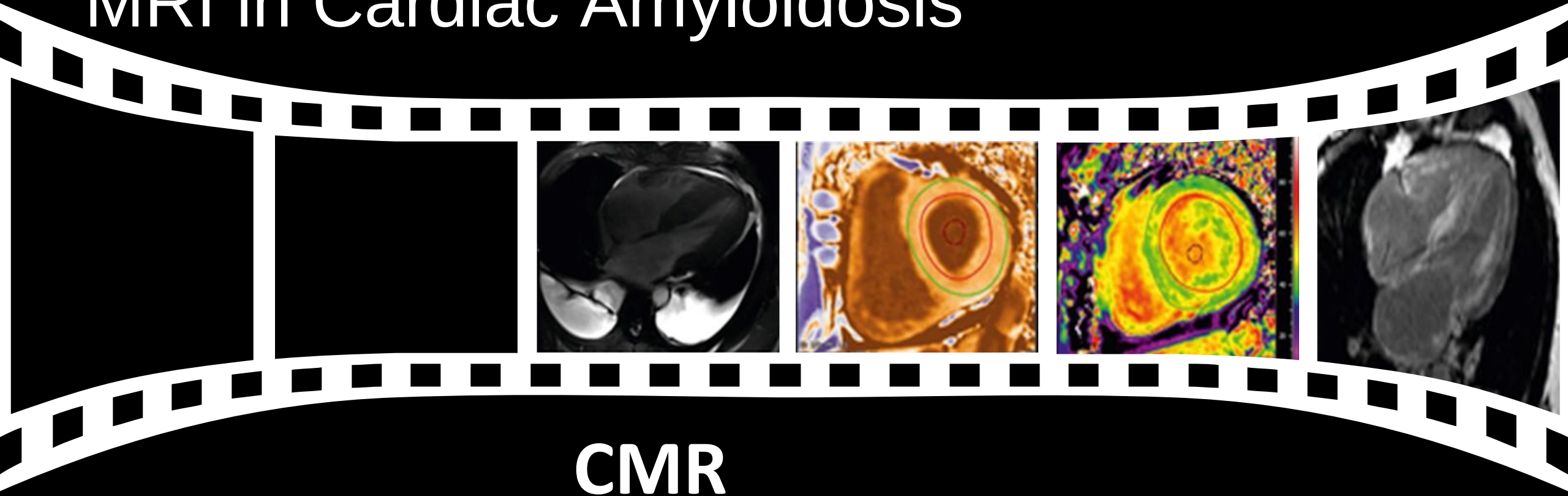
28.7%

17.9%

10.3%



MRI in Cardiac Amyloidosis

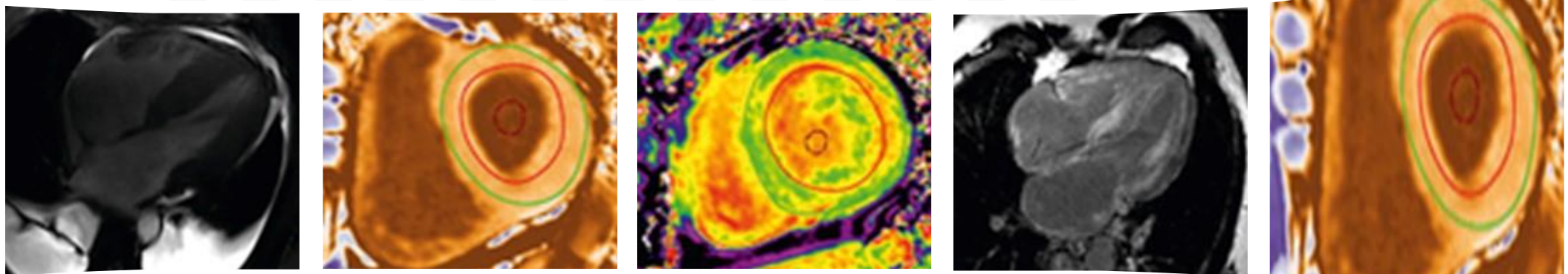


CMR

Thickend walls

Preseved function

MRI in Cardiac Amyloidosis



Native T1

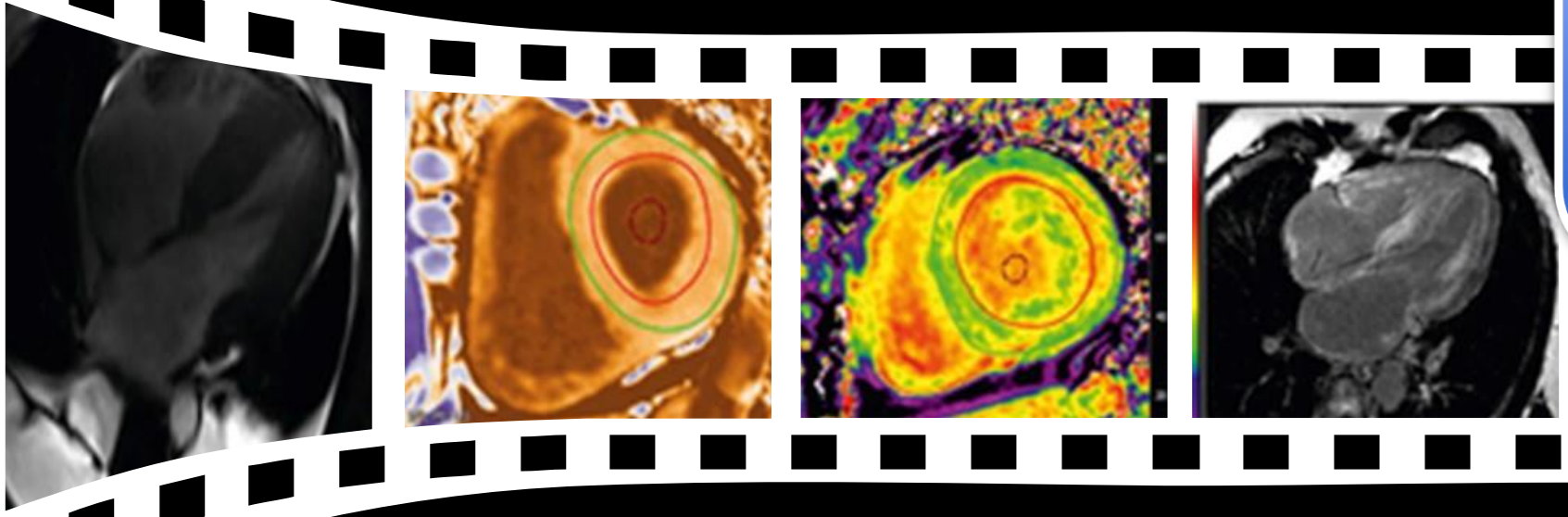
Numerical time parameter (ms)

Native T1 times are higher in patients with

- Amyloid (1409 ± 59 ms, $p < 0.0001$) compared to
- Healthy controls (1225 ± 21 ms),
- HCM (1266 ± 44 ms)

MRI in Cardiac Amyloidosis

25%

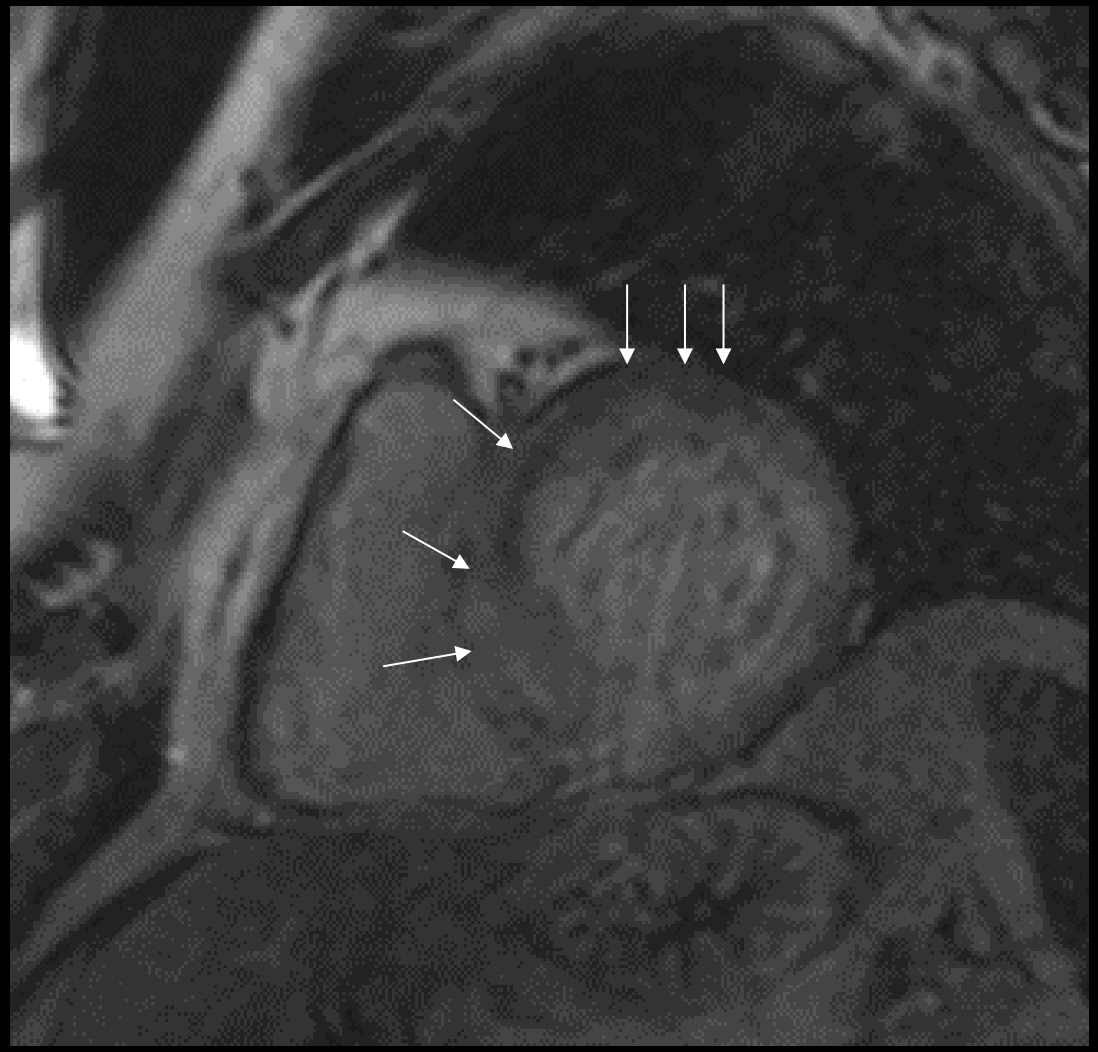


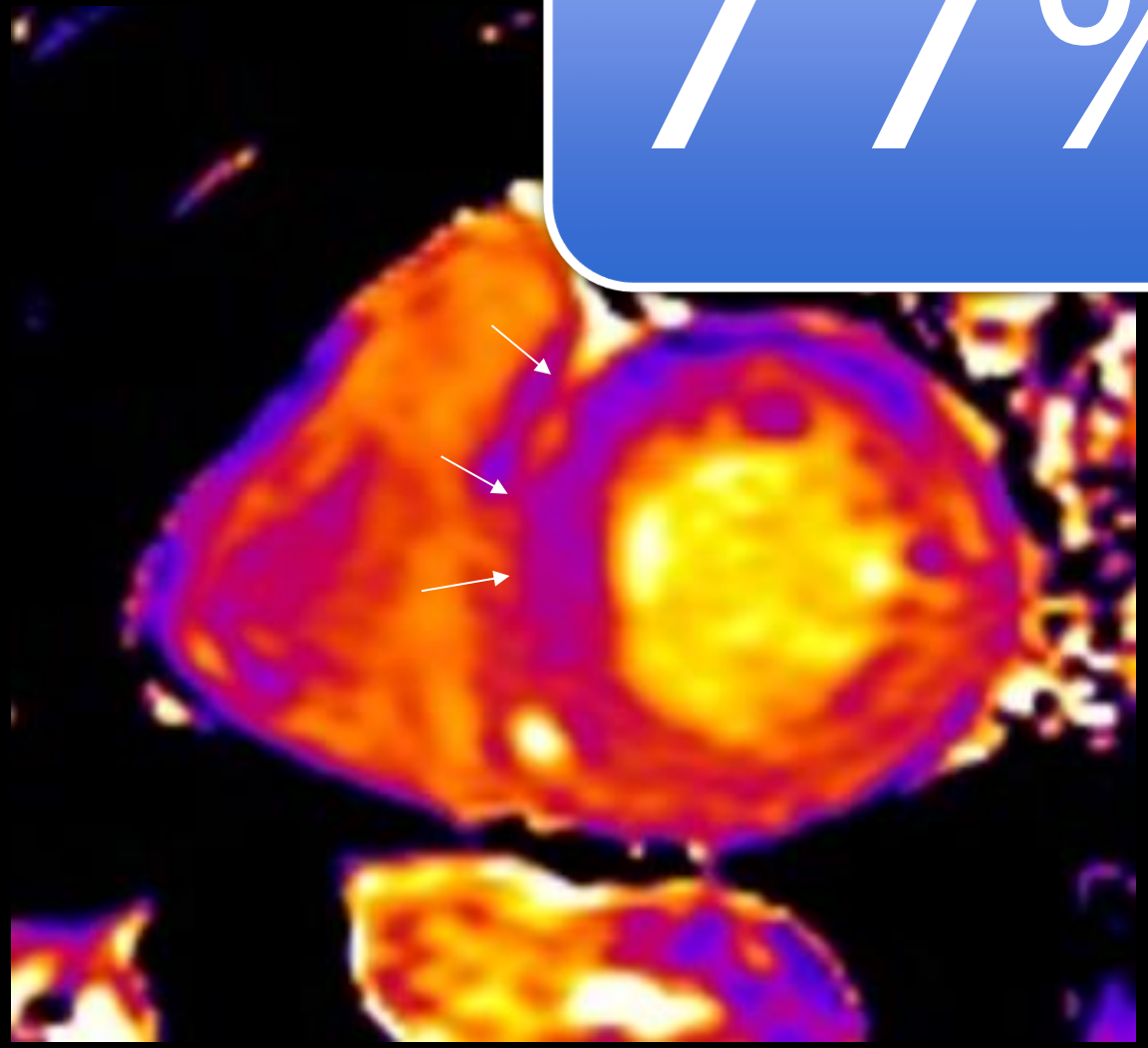
Extracellular water volume ECV

More protein = more water

- Calculated from ECV_{CMR} = (1–haematocrit) × ($\Delta(1/T1_{myo})/\Delta(1/T1_{blood})$)
- Normal ECV values of 25.3±3.5% in normal myocardium

Amyloid – MRI ECV mapping



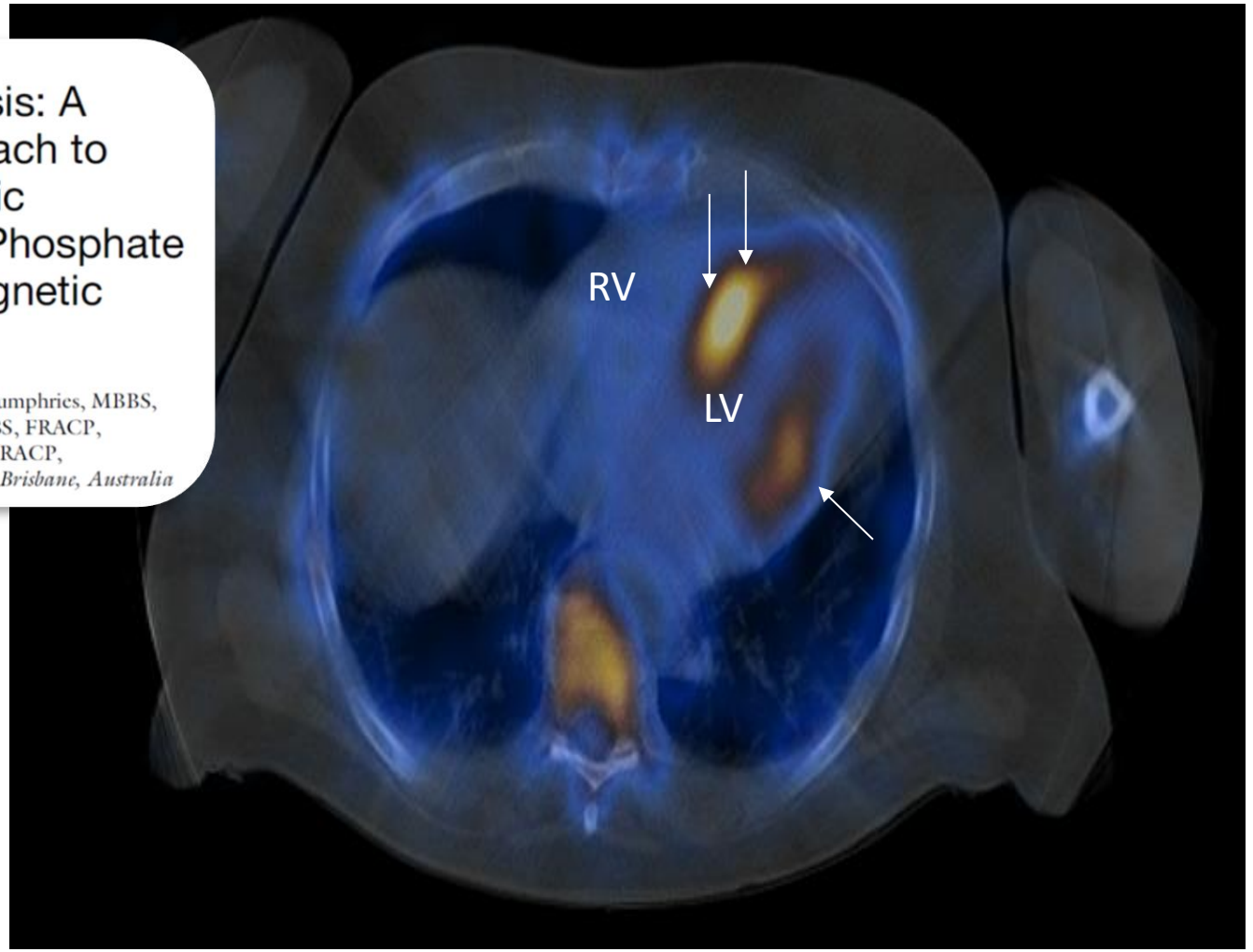


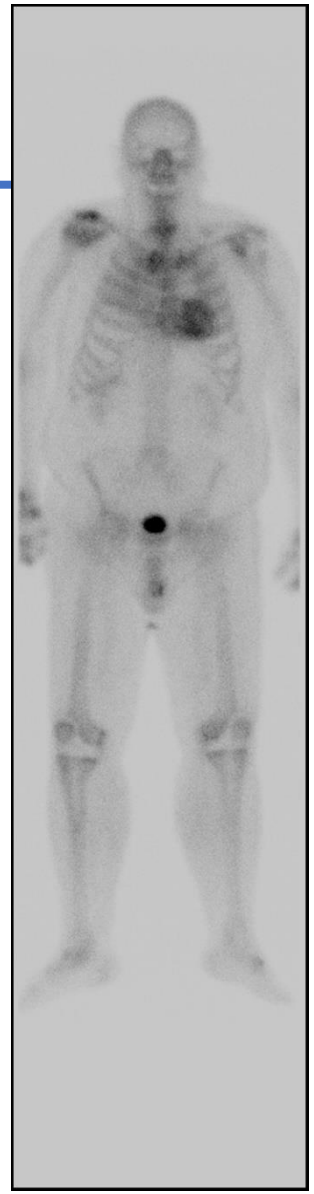
77%

Amyloid – TTR Bone Scan

Transthyretin Cardiac Amyloidosis: A Noninvasive Multimodality Approach to Diagnosis Using Transthoracic Echocardiography, 99m-Tc-Labeled Phosphate Bone Scanning, and Cardiac Magnetic Resonance Imaging

Akhil Shukla, MB BCh, BSci, David Wong, MBBS, RANZACR, Julie A. Humphries, MBBS, BHMS(Ed), FRACP, FCSANZ, FASE, Benjamin T. Fitzgerald, MBBS, FRACP, Katrina Newbigin, MBBS, RANZACR, John Bashford, MBBS, FRACP, and Gregory M. Scalia, MBBS, MMedSci, FRACP, FCSANZ, FACC, FASE, *Brisbane, Australia*





CASE: Cardiovascular Imaging Case Reports
April 2017

Perugini Criteria

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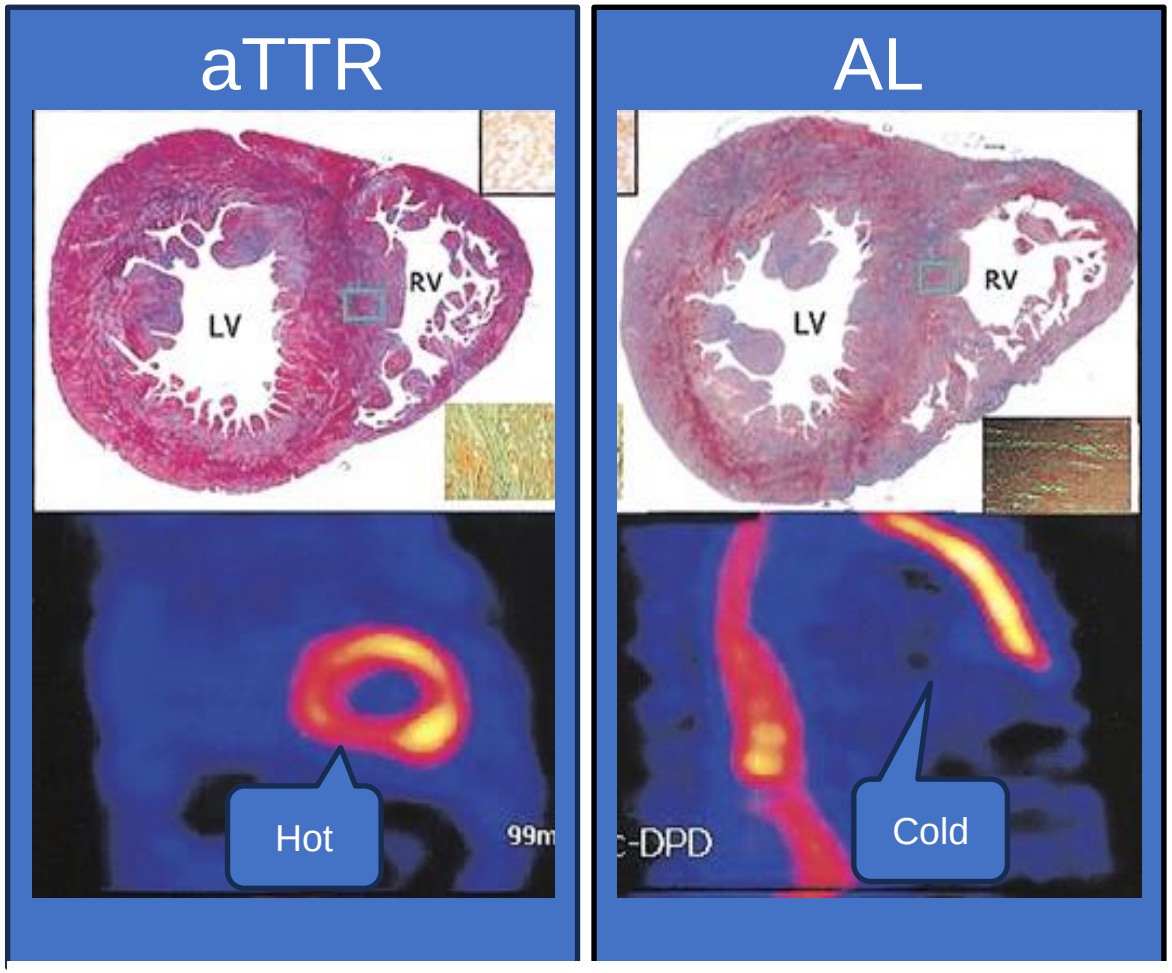
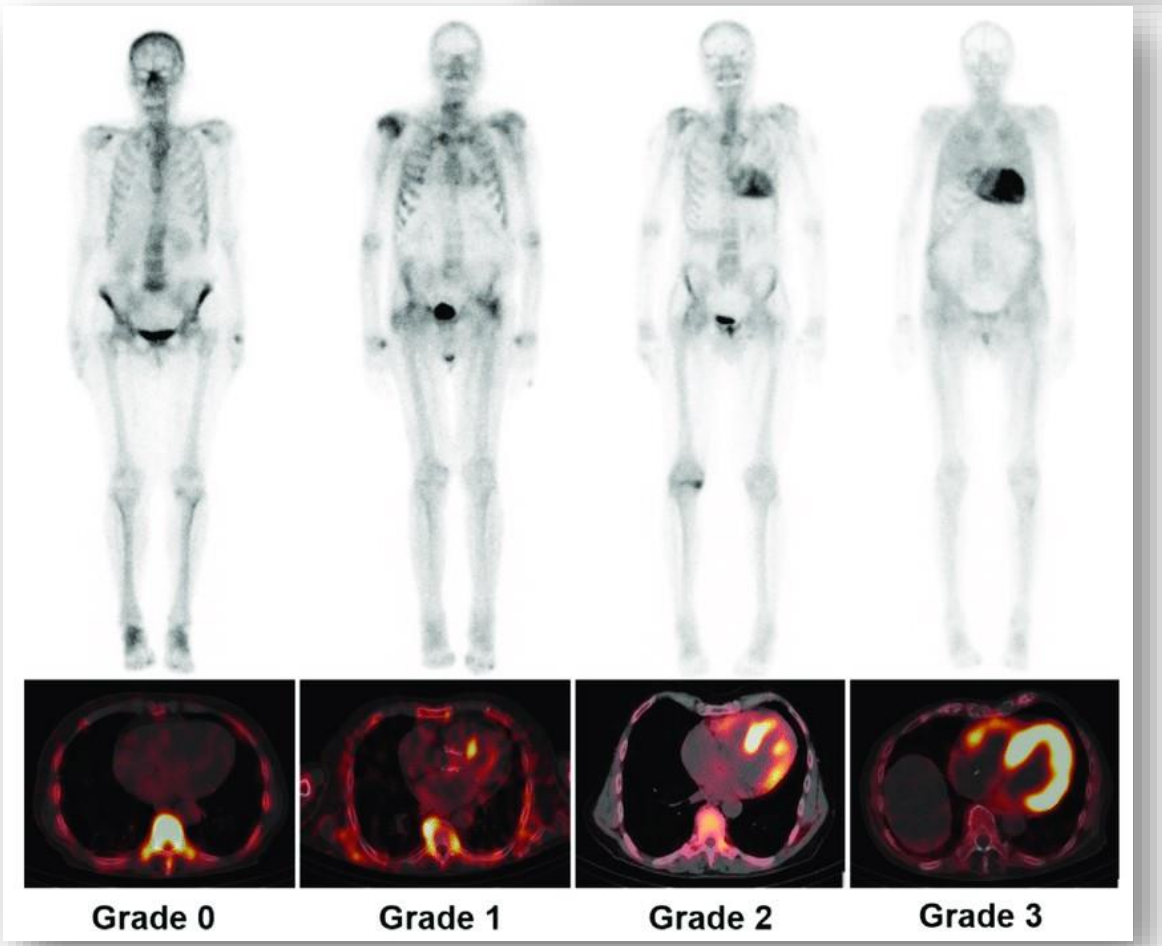
ISSN 0735-1097/05/$30.00
doi:10.1016/j.jacc.2005.05.073

Cardiac Imaging

Noninvasive Etiologic Diagnosis of Cardiac Amyloidosis Using ^{99m}Tc-3,3-Diphosphono-1,2-Propanodicarboxylic Acid Scintigraphy

Enrica Perugini, MD,* Pier Luigi Guidalotti, MD,† Fabrizio Salvi, MD,‡ Robin M. T. Cooke, MA,* Cinzia Pettinato, MD,† Letizia Riva, MD,* Ornella Leone, MD,§ Mohsen Farsad, MD,† Paolo Ciliberti, MD,* Letizia Bacchi-Reggiani, MSc, MBiostat,* Francesco Fallani, MD,* Angelo Branzi, MD,* Claudio Rapezzi, MD*
Bologna, Italy





Grade 0: unaffected individual without any cardiac tracer uptake
Grade 1: patient with AL and mild cardiac uptake
Grade 2: patient with ATTR and strong cardiac uptake greater than bone uptake
Grade 3: patient with ATTR and pronounced tracer uptake in the myocardium but reduced bone uptake.

ECHO AUSTRALIA

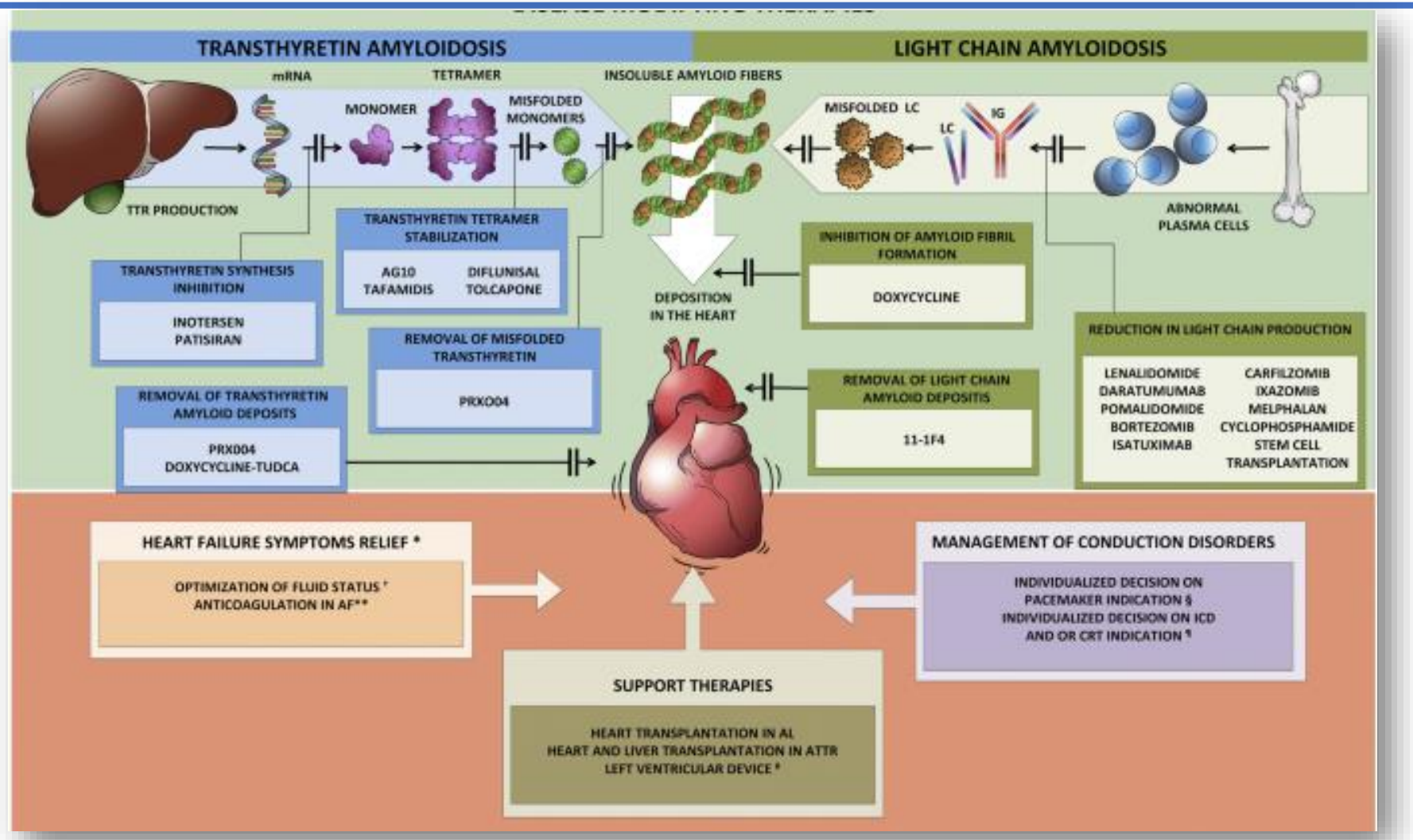
Heart, Lung and Circulation (2024) 33, 420–442
1443-9506/24/$36.00
https://doi.org/10.1016/j.hlc.2023.11.027

ORIGINAL ARTICLE

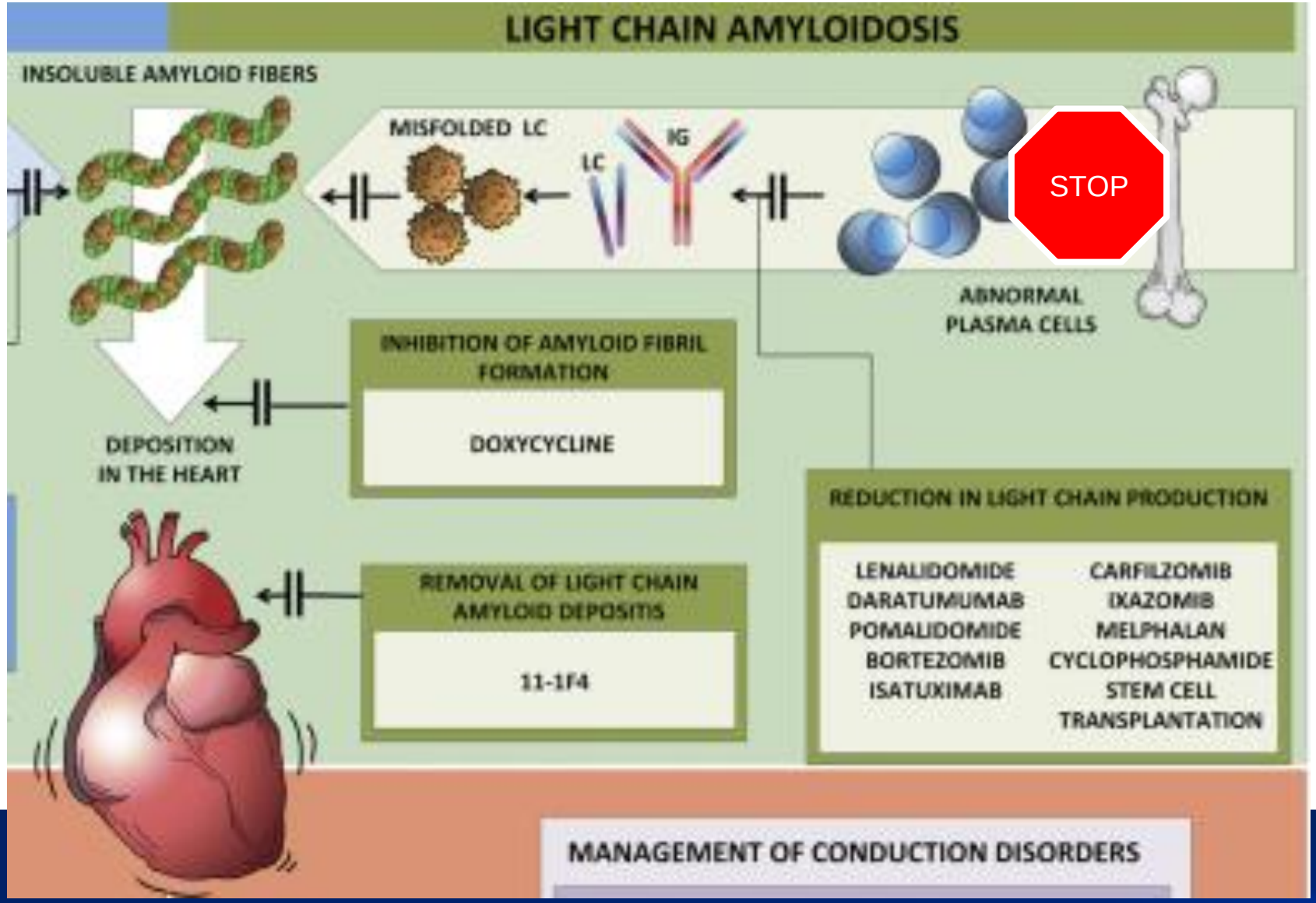
2024 Australia–New Zealand Expert Consensus Statement on Cardiac Amyloidosis

Nicole K. Bart, MBBS, DPhil [a], Diane Fatkin, BSc(Med), FRACP, FSCANZ [a],
James Gunton, MBBS, PhD [b], James L. Hare, PhD, FRACP [c],
Dariusz Korczyk, FRACP, FCSANZ, DDU [d], Fiona Kwok, PhD, FRACP [e],
Kaitlyn Lam, MBBS, PhD [f], David Russell, BMedSci, FRACP, FCSANZ [g],
Hasib Sidiqi, MBBS, PhD [h], Tim Sutton, FRACP, FCSANZ [i],
Simon D.J. Gibbs, FRACP, FRCPA [j],
Peter Mollee, MMedSc, FRACP, FRCPA [k], Liza Thomas, MBBS, PhD [l,*]

Treatment of amyloidosis



Treatment of AL amyloidosis



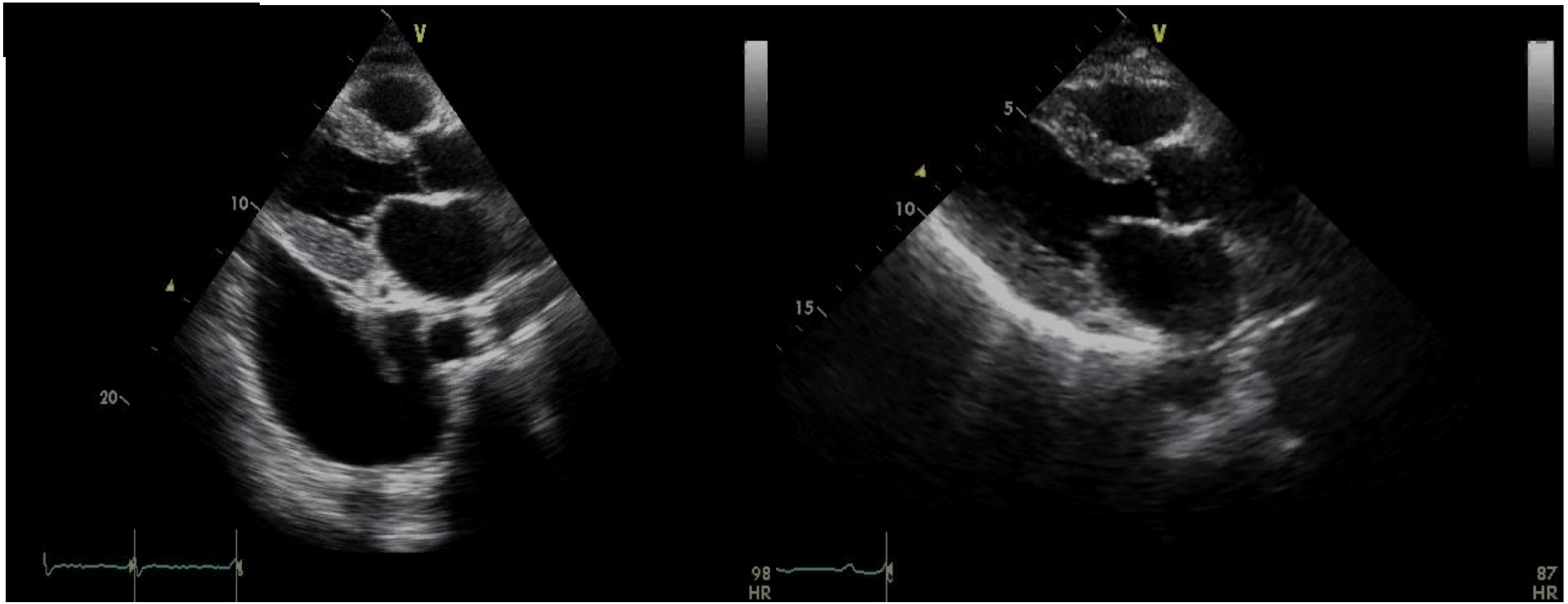
Treatment of AL amyloidosis

- Peripheral Stem Cell Transplant
  - Harvest cell from blood (not marrow)
  - High Dose chemotherapy

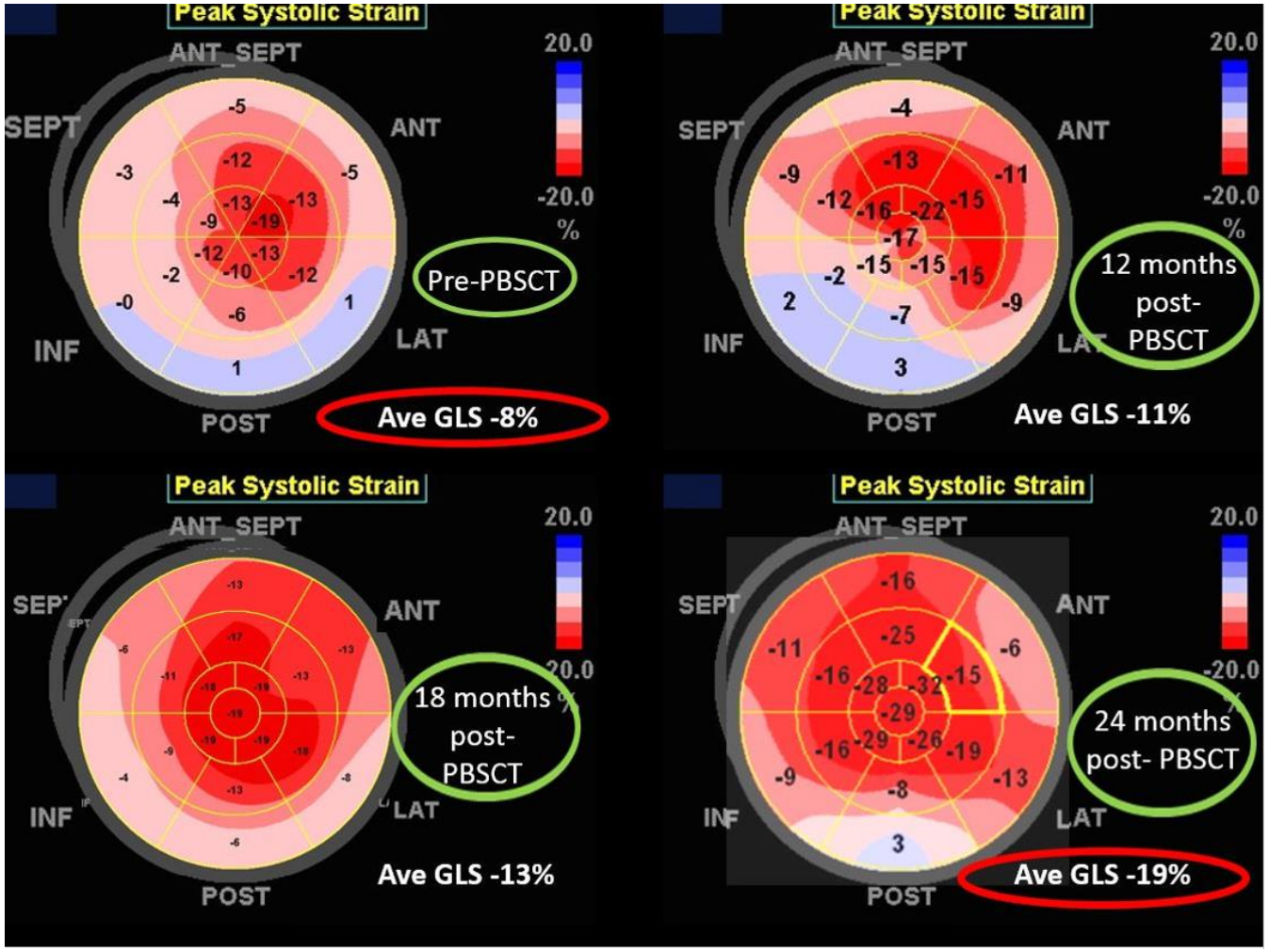
  And/or
  - Modern treatments now use
    - Bortezomib (Velcade)
    - Thalidomide-type drugs
- Marked reduction in cell clone numbers/paraprotein excretion

Stem cell concentrate

AL - Amyloid cell transplant



Regression of AL amyloidosis



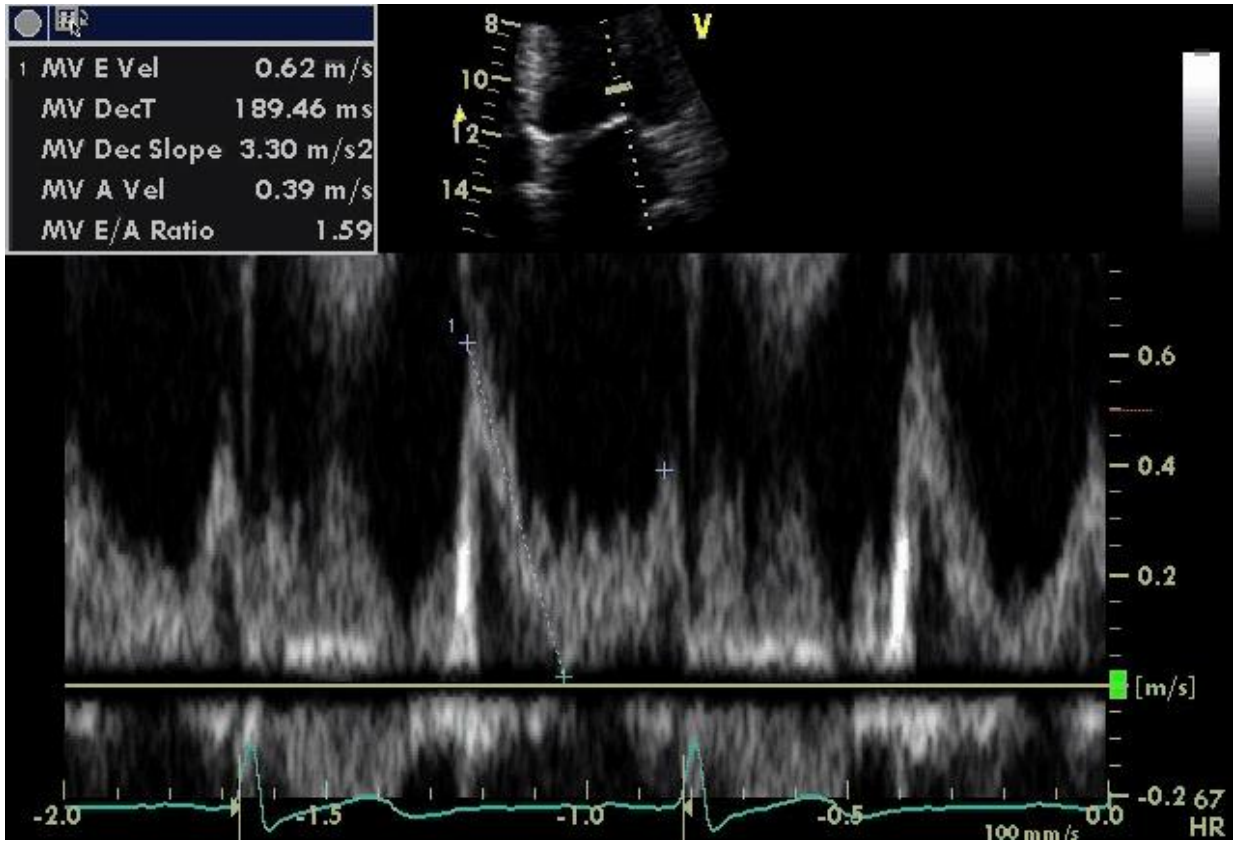
Regression of the Anatomic Cardiac Features of Amyloid Light Chain Cardiac Amyloidosis Accompanied by Normalization of Global Longitudinal Strain

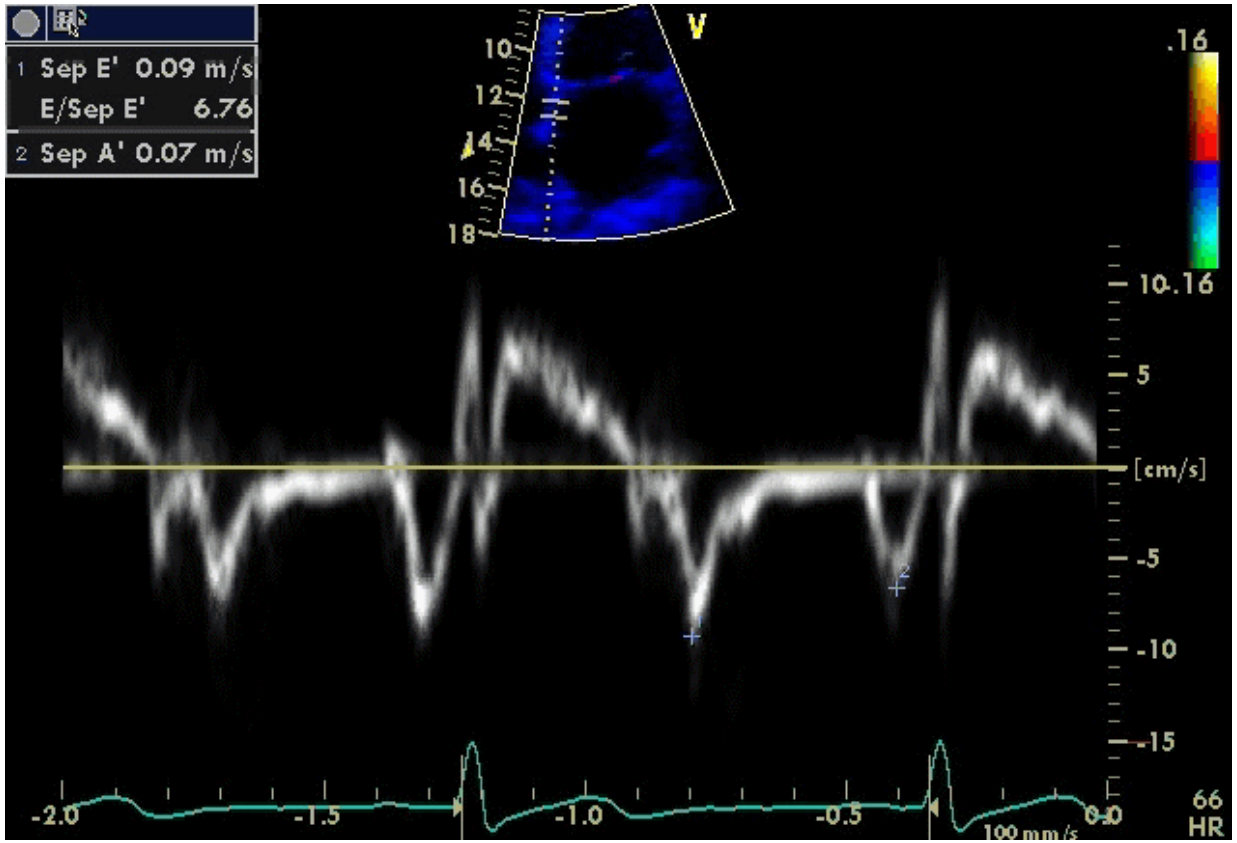
Benjamin T. Fitzgerald, MBBS, John Bashford, MBBS, and Gregory M. Scalia, MBBS,
Auchenflower, Queensland, Australia

CASE: Cardiovascular Imaging Case Reports
Volume 1 Number 2

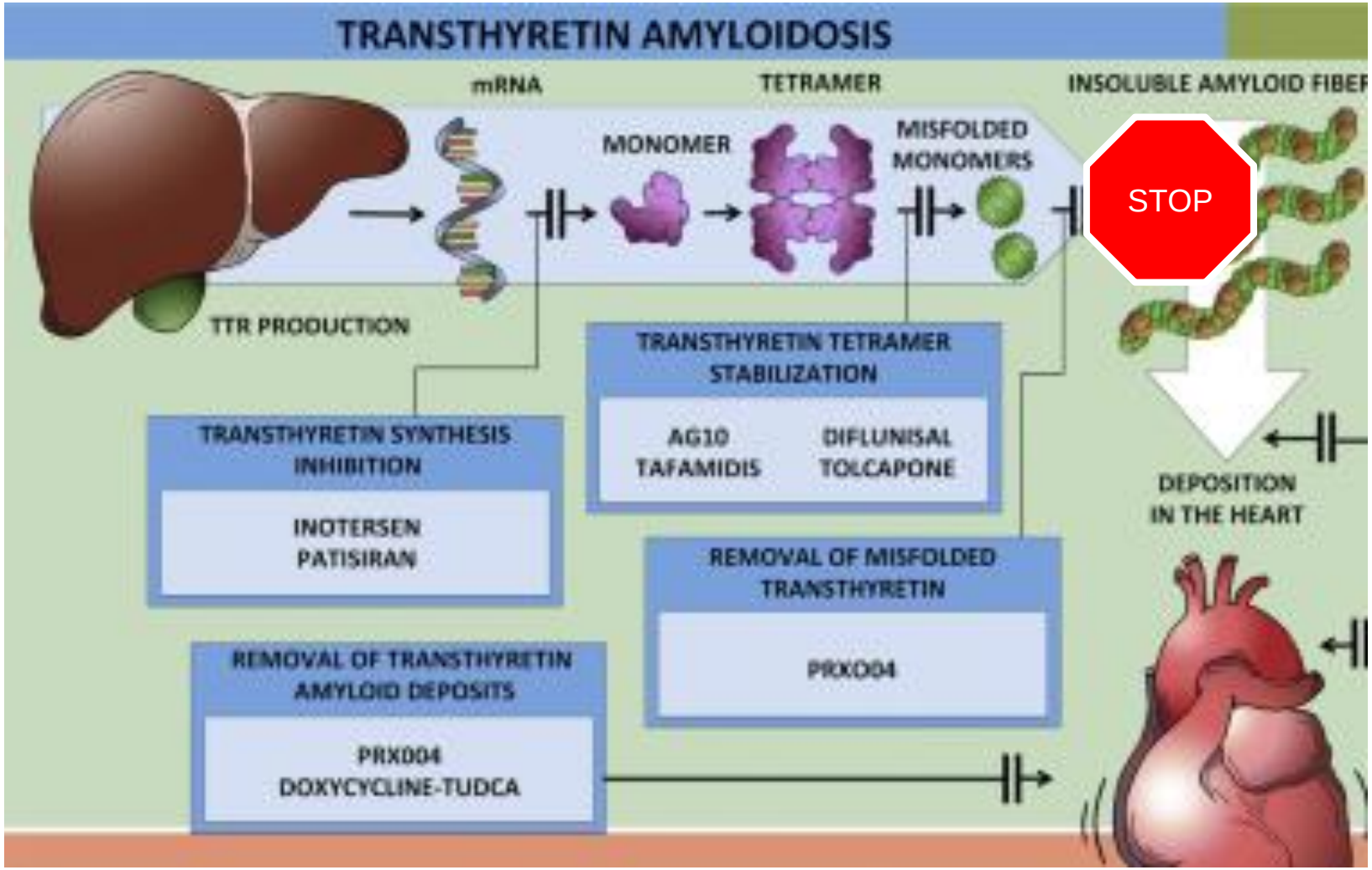
Regression of AL amyloidosis

Deceleration time 189ms
E/e’ = 7
Normal diastolic function



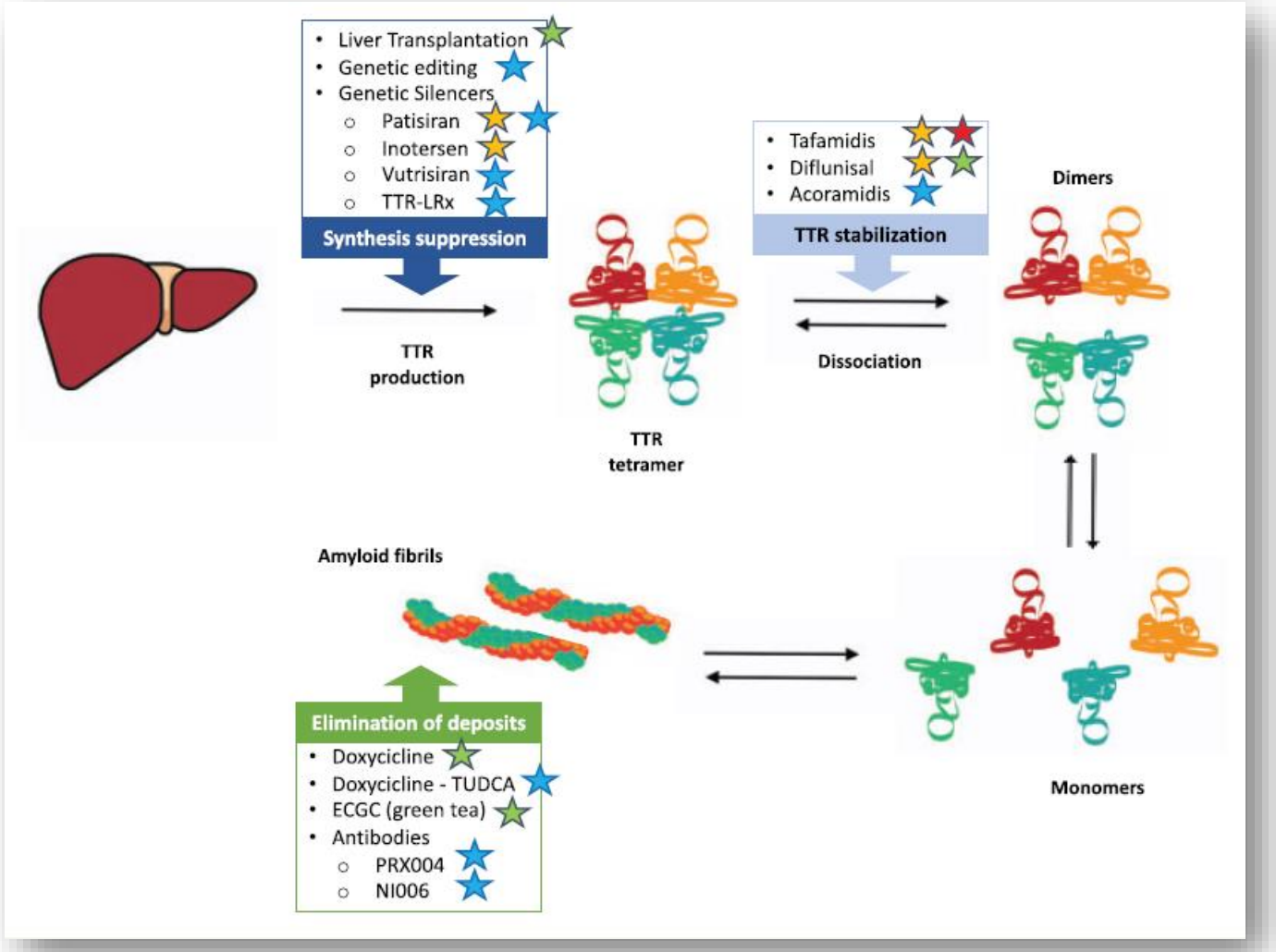


Treatment of TTR amyloidosis

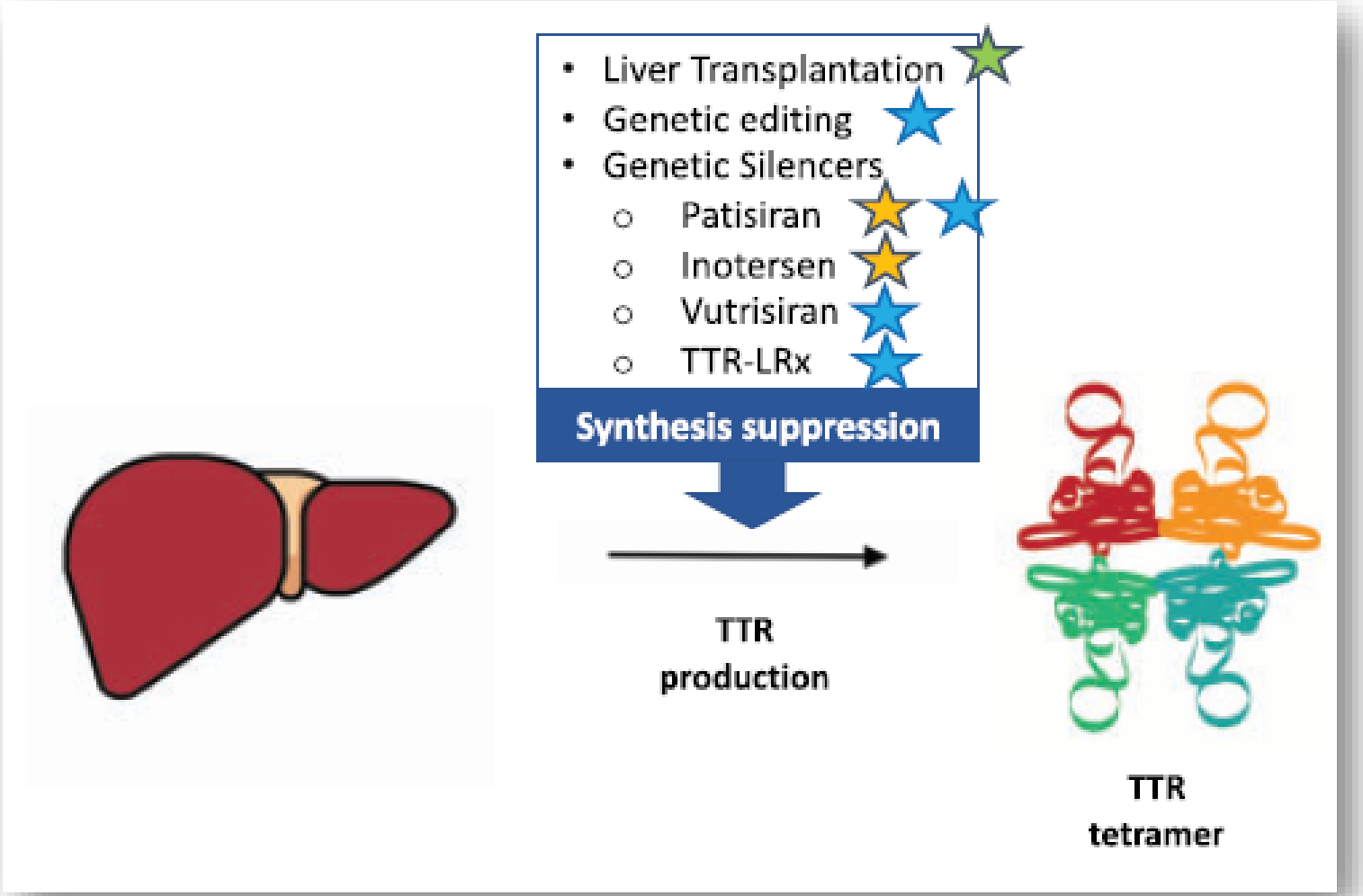


European Heart Journal (2021) **00**, 1–15

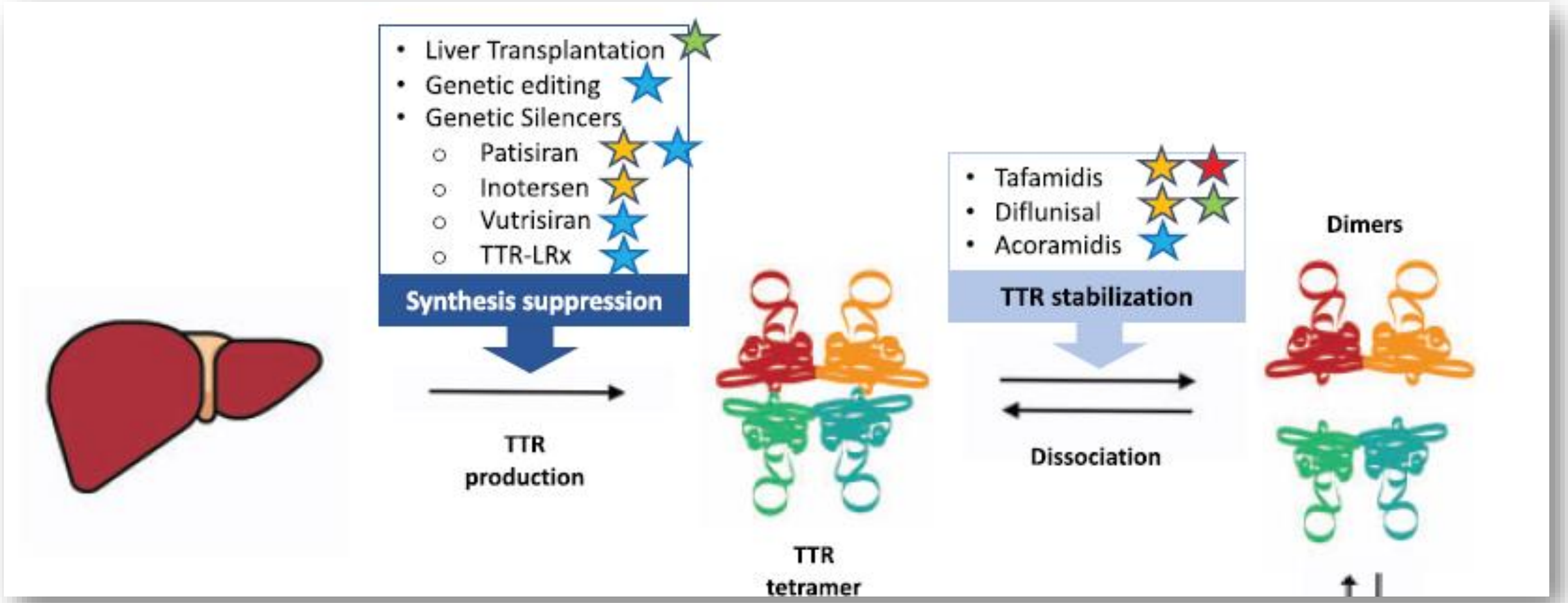
Available and future disease-modifying Rx for aTTR



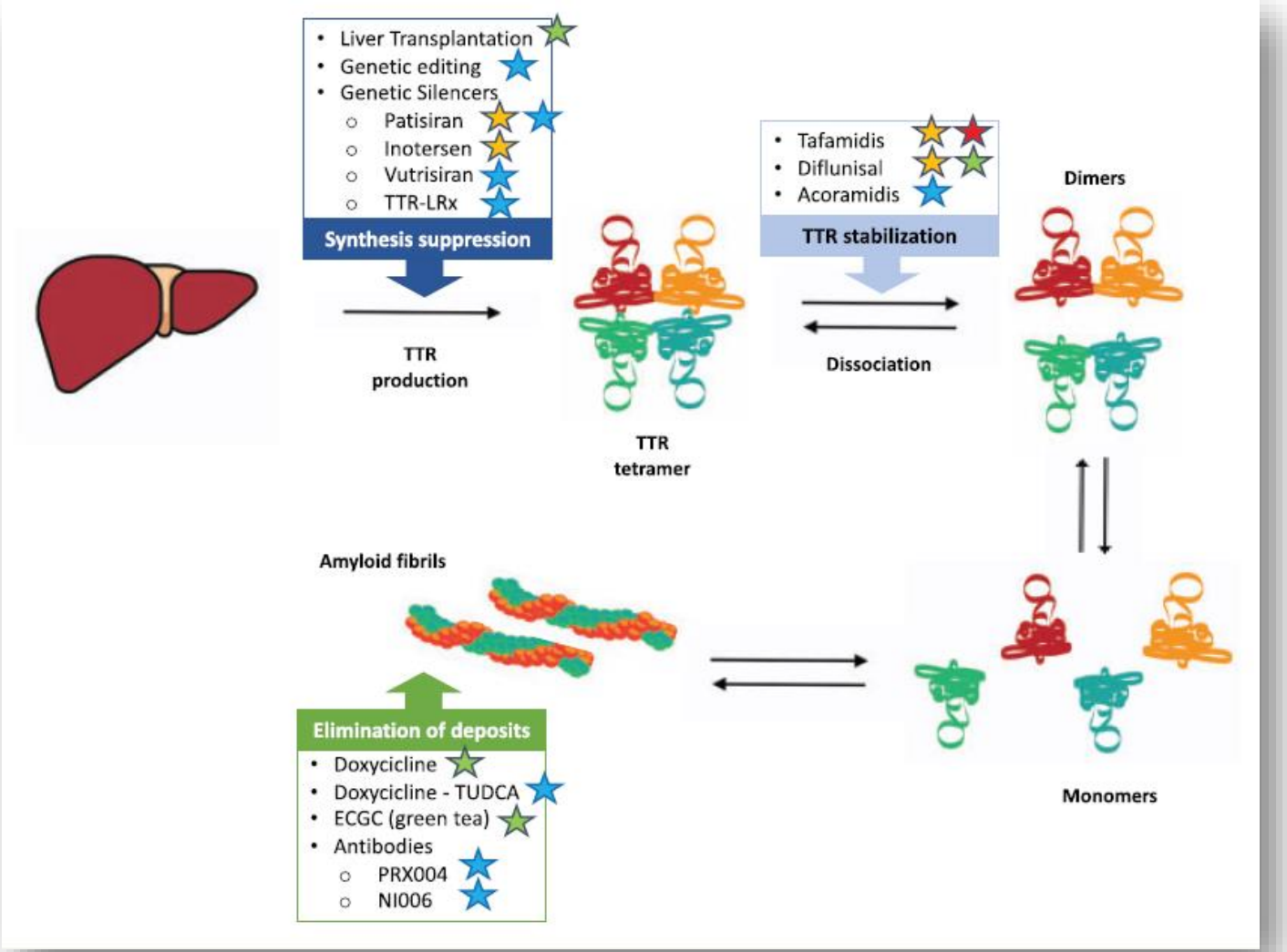
Available and future disease-modifying Rx for aTTR



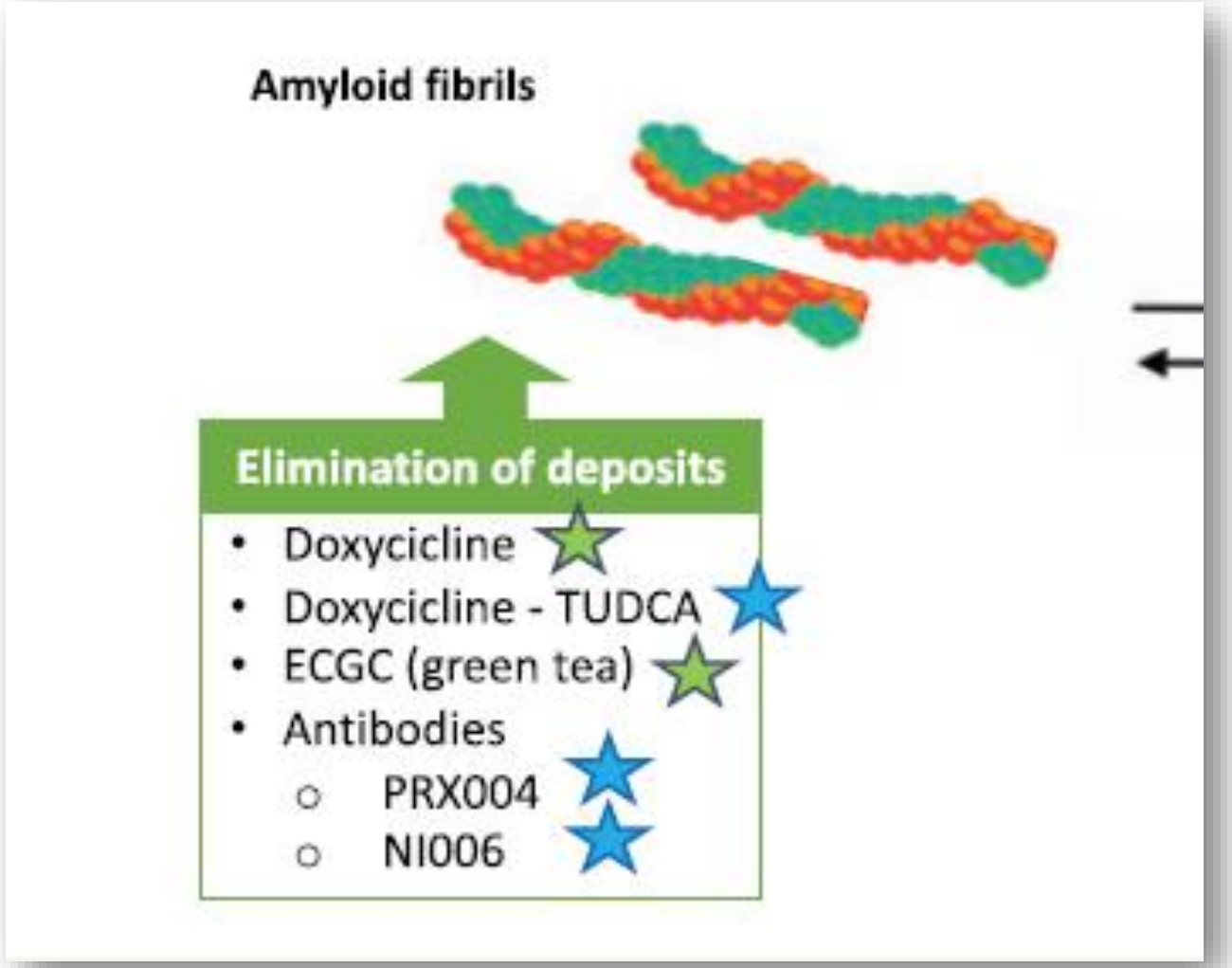
Available and future disease-modifying Rx for aTTR



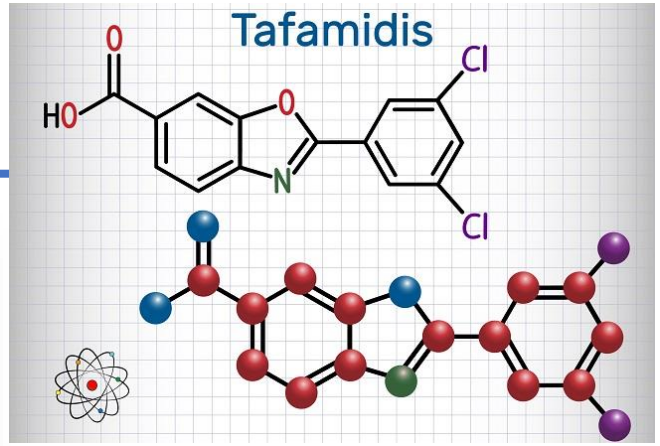
Available and future disease-modifying Rx for aTTR



Available and future disease-modifying Rx for aTTR



TTR Amyloid Treatment

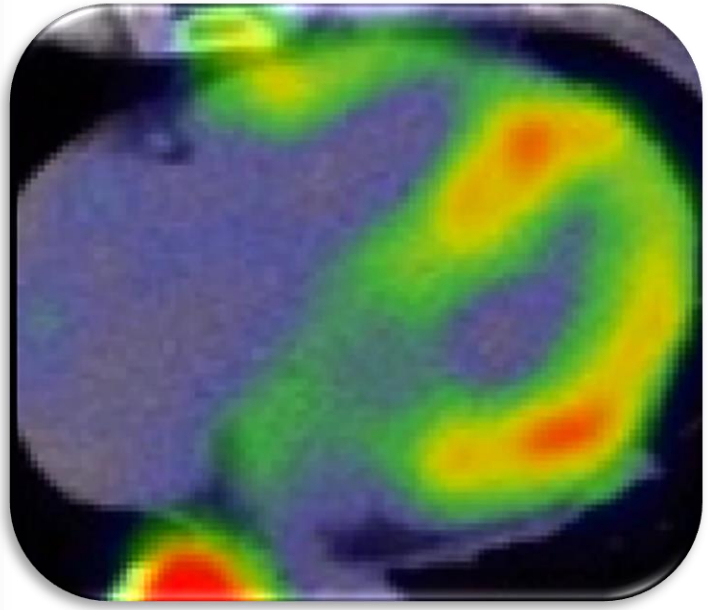


The NEW ENGLAND JOURNAL of MEDICINE

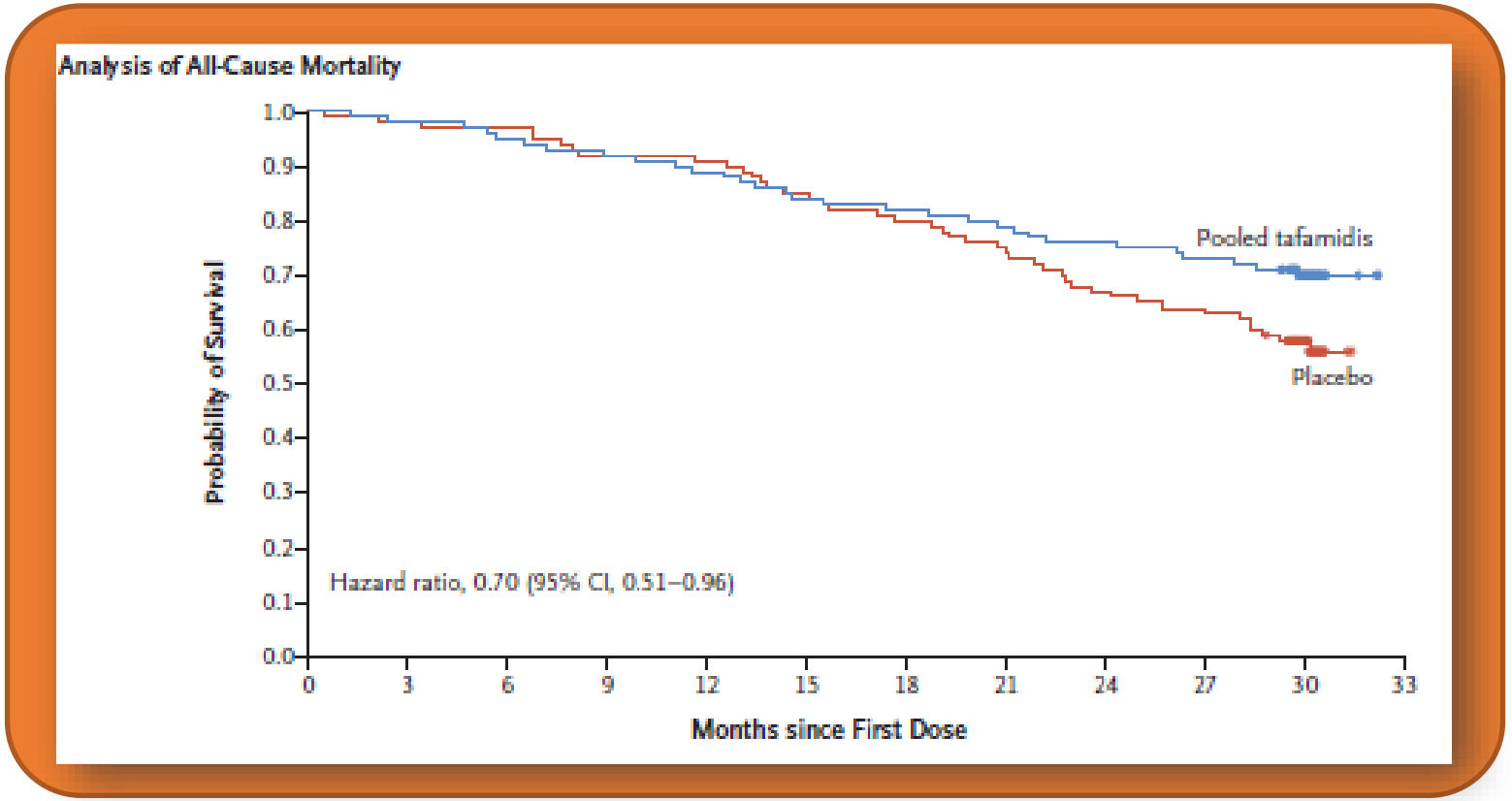
ESTABLISHED IN 1812 SEPTEMBER 13, 2018 VOL. 379 NO. 11

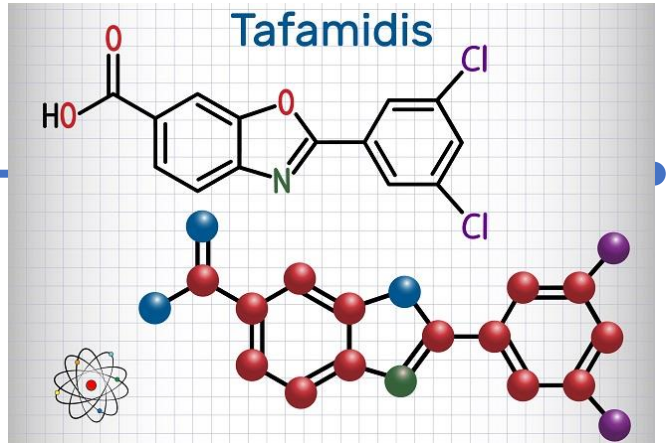
Tafamidis Treatment for Patients with Transthyretin Amyloid Cardiomyopathy

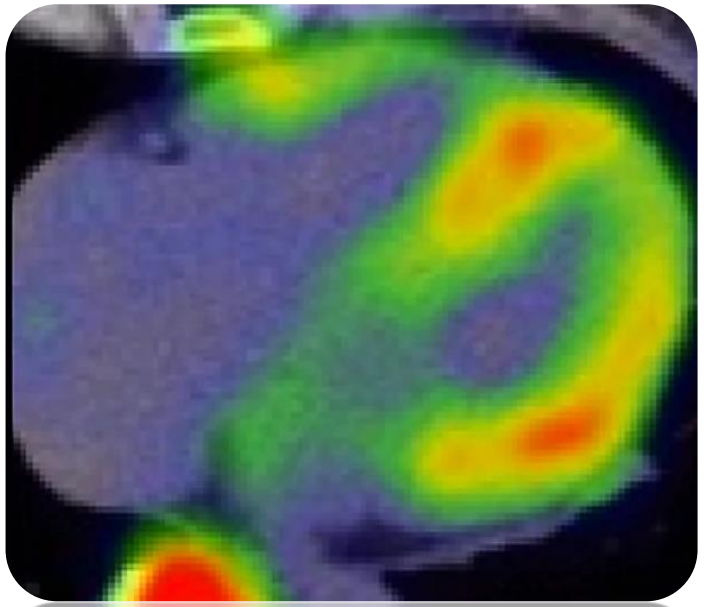
Mathew S. Maurer, M.D., Jeffrey H. Schwartz, Ph.D., Balarama Gundapaneni, M.S., Perry M. Elliott, M.D., Giampaolo Merlini, M.D., Ph.D., Marcia Waddington-Cruz, M.D., Arnt V. Kristen, M.D., Martha Grogan, M.D., Ronald Witteles, M.D., Thibaud Damy, M.D., Ph.D., Brian M. Drachman, M.D., Sanjiv J. Shah, M.D., Mazen Hanna, M.D., Daniel P. Judge, M.D., Alexandra I. Barsdorf, Ph.D., Peter Huber, R.Ph., Terrell A. Patterson, Ph.D., Steven Riley, Pharm.D., Ph.D., Jennifer Schumacher, Ph.D., Michelle Stewart, Ph.D., Marla B. Sultan, M.D., M.B.A., and Claudio Rapezzi, M.D., for the ATTR-ACT Study Investigators*



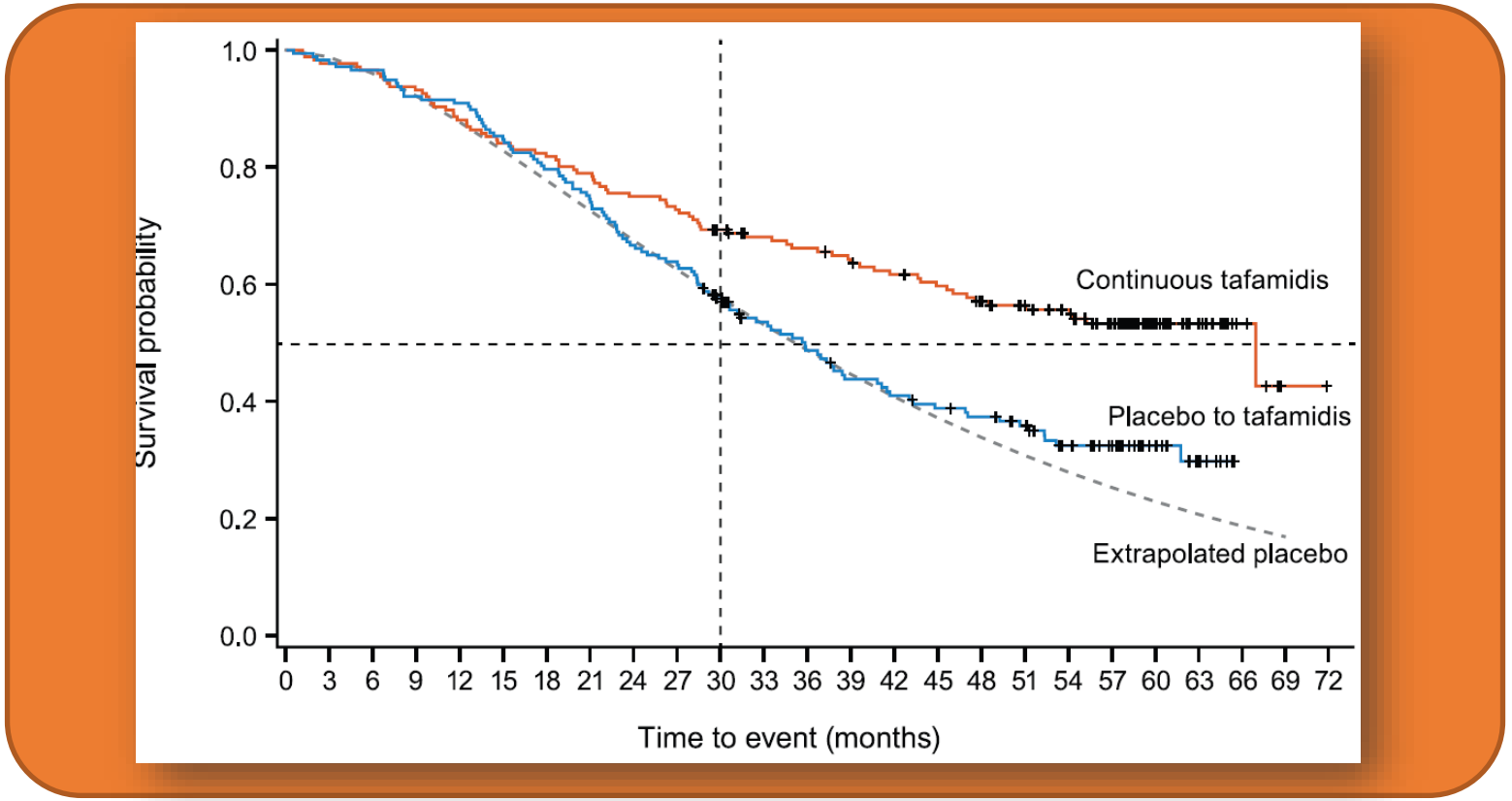
TTR Amyloid Treatment

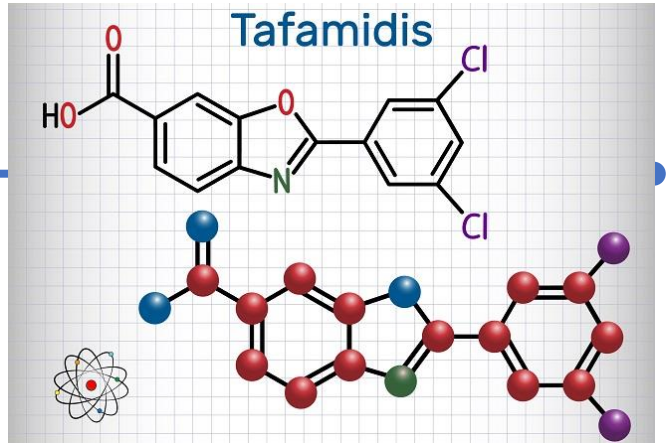


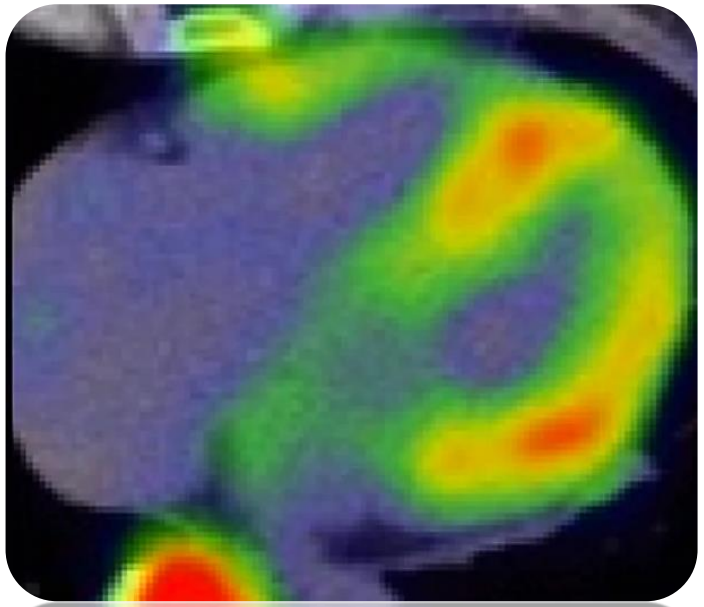




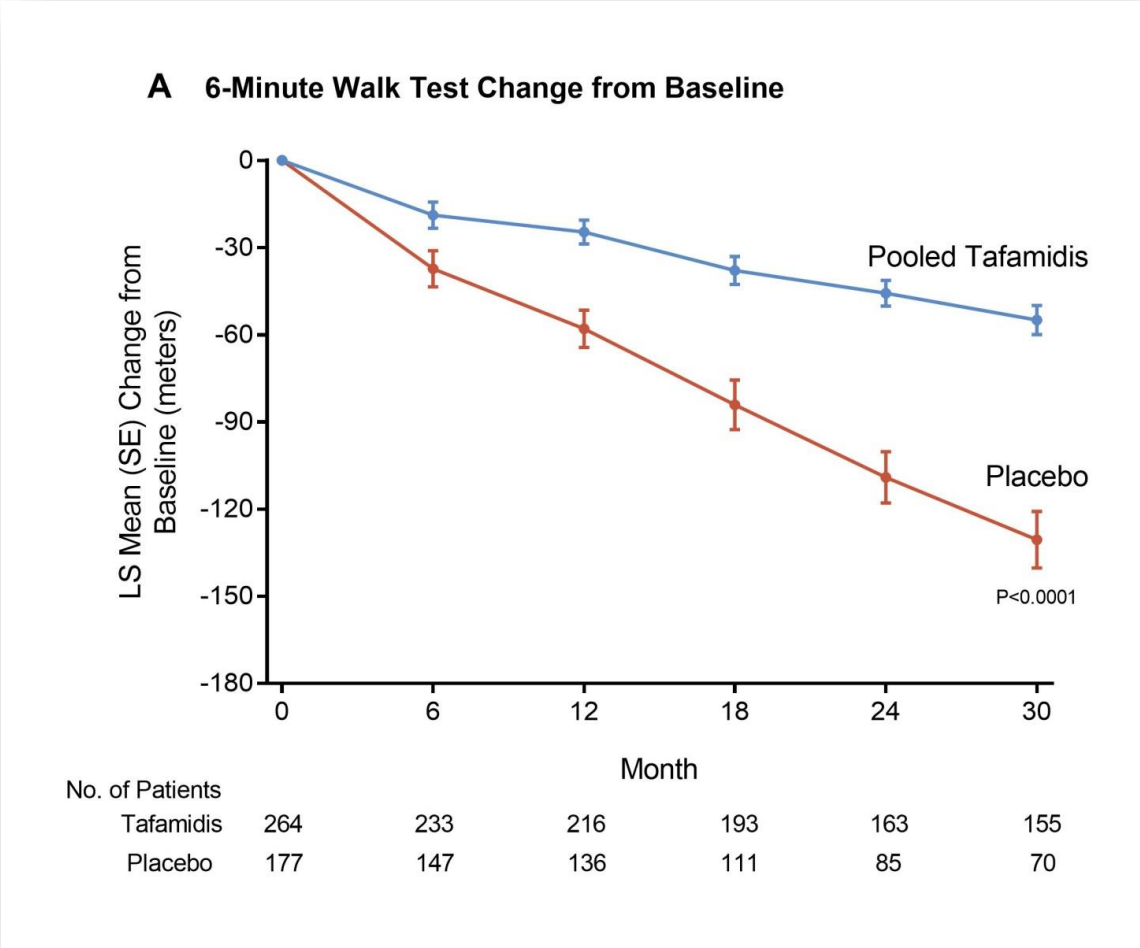
TTR Amyloid Treatment

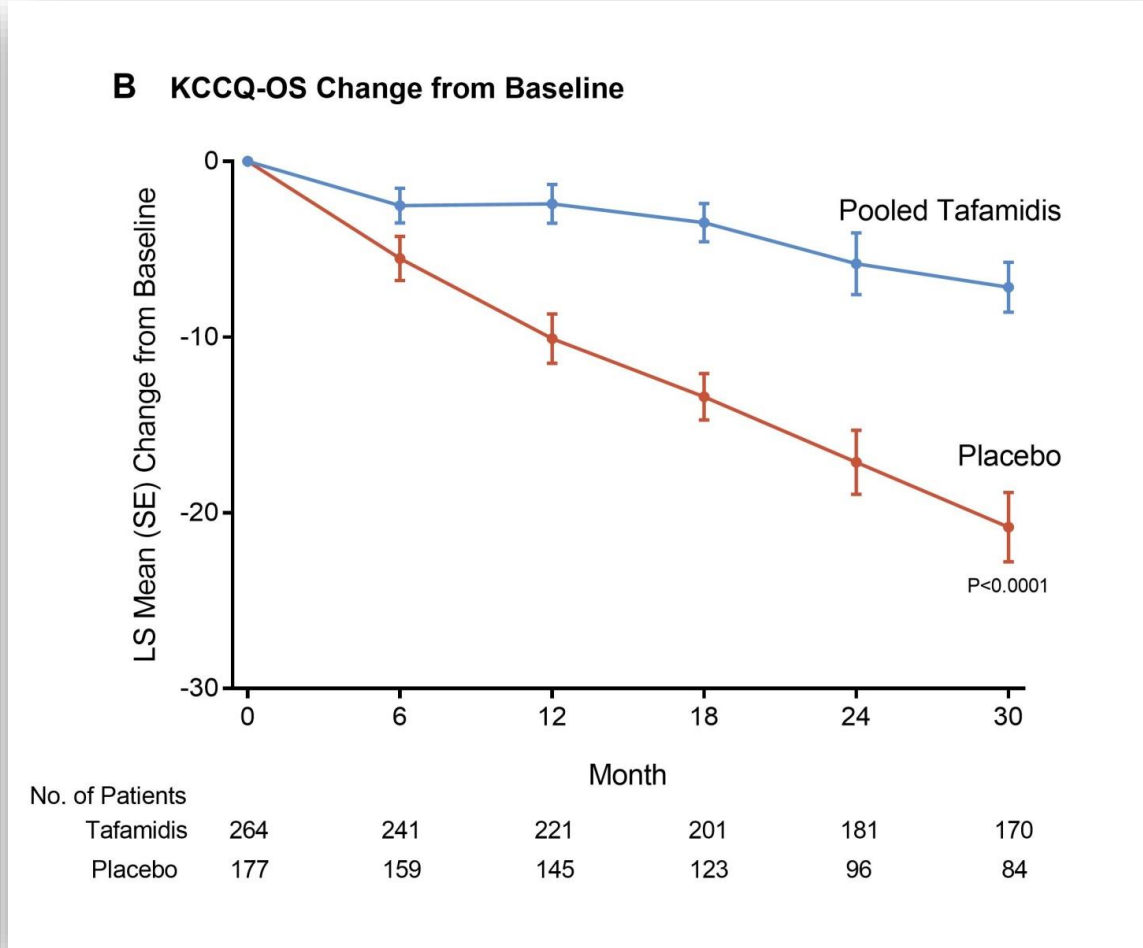






Tafamidis reduces the decline in the 6MWT distance and KCCQ-OS score at 30 months





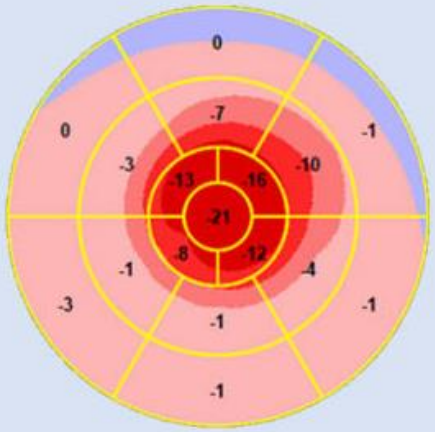
Maurer M et al , New England Journal of Medicine 2018

Effect of tafamidis on global longitudinal strain and myocardial work in aTTR

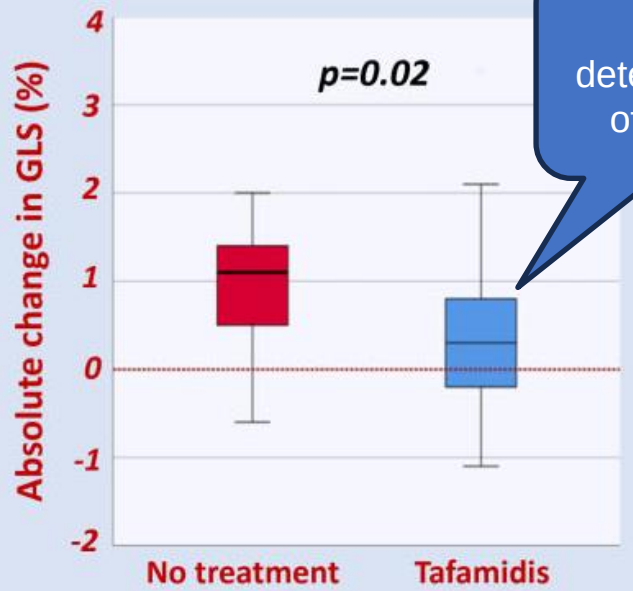
Transthyretin amyloid cardiomyopathy

23 patients treated with tafamidis

22 patients untreated



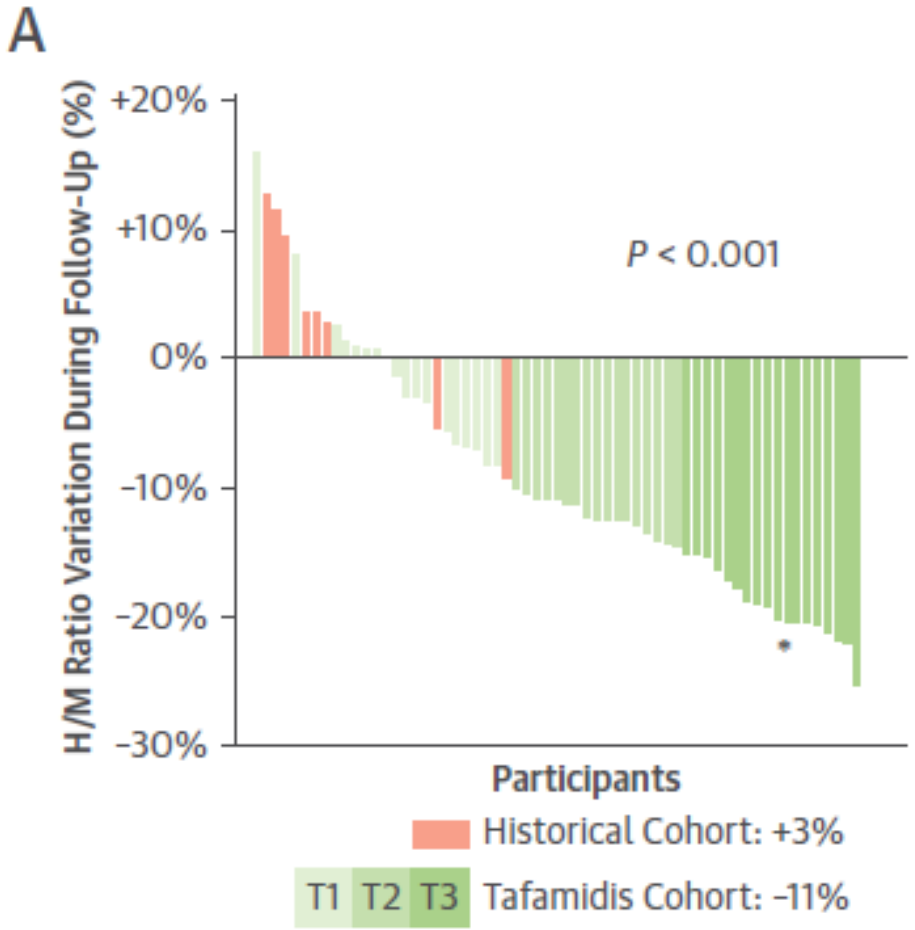
Speckle tracking echocardiography and myocardial work assessment at baseline and 1 year

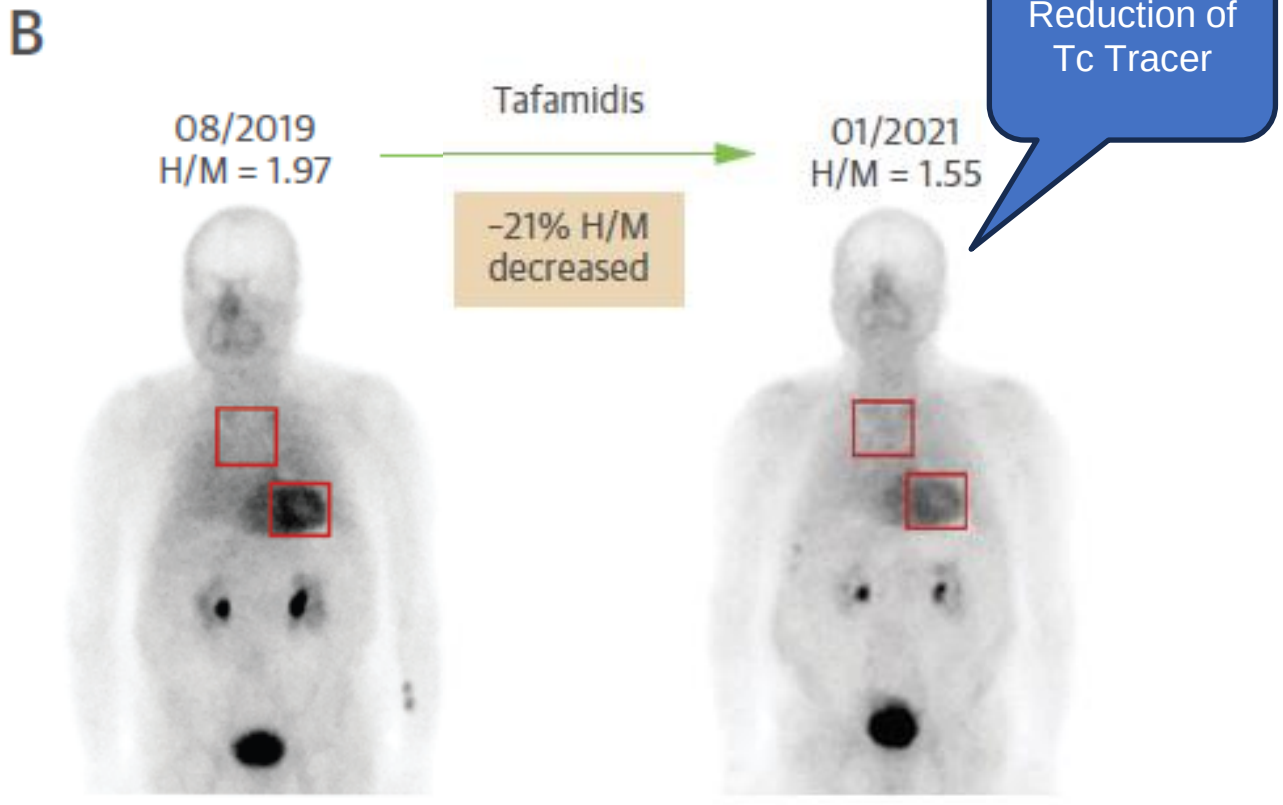


Less deterioration of Strain

Lesser deterioration in GLS, myocardial work index and myocardial efficiency over 12 months in those treated with tafamidis

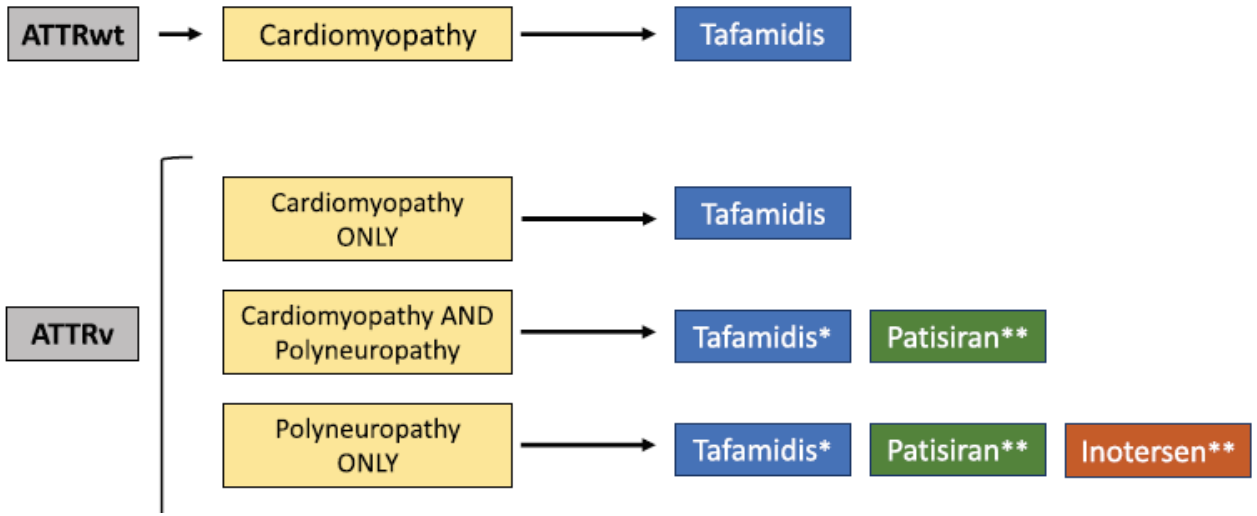
Tafamidis Decreases Cardiac Uptake of 99mTc-HMDP in ATTR





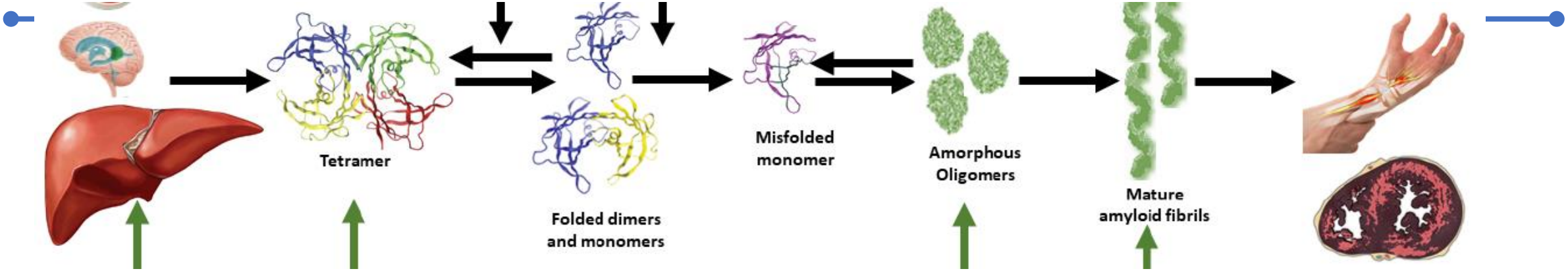
ESC Guidelines

Recommendations	Class[a]	Level[b]
Tafamidis is recommended in patients with genetic testing proven hereditary hTTR-CMP and NYHA class I or II symptoms to reduce symptoms and CV hospitalization and mortality.	I	B
Tafamidis is recommended in patients with wtTTR-CA and NYHA class I or II symptoms to reduce symptoms and CV hospitalization and mortality.	I	B



European Heart Journal (2021) **00**, 1–15

Mechanisms of action of ATTR-CM new treatments



- Liver Transplantation
- TTR Gene Silencing
  - -siRNA and ASO : Patisiran, Revusiran, Vutrisiran, Inotersen, Eplontersen
  - -Crispr Cas 9

- TTR Stabilizers
  - Tafamidis,
  - Acoramidis,
  - Diflunisal

Adapted from Carroll A, et al. J Neurol Neurosurg P 2022

ECHO AUSTRALIA

Undergoing Clinical Trial including ATTR-WT

Drugs	Patisiran	Vutrisiran	ION-682884
Inclusion	**ATTR Cardiomyopathy**	**ATTR Cardiomyopathy**	**ATTR Cardiomyopathy**
Trial	**Phase 3 APPOLO-B : Just published**	**Phase 3 HELIOS B Undergoing 2024**	**Phase 3 CARDIO TTRansform Undergoing 2025**
Methodology	Phase 3 / Double blind	Phase 3 / Double blind	Phase 3 / Double blind
Administration	Intravenous	Subcutaneous	Subcutaneous
Where we are?	Inclusion started	Inclusion started	Inclusion pending

Patisiran

The NEW ENGLAND JOURNAL of MEDICINE

ORIGINAL ARTICLE FREE PREVIEW

Patisiran Treatment in Patients with Transthyretin Cardiac Amyloidosis

Mathew S. Maurer, M.D., Parag Kale, M.D., Marianna Fontana, M.D., Ph.D., John L. Berk, M.D., Martha Grogan, M.D., Finn Gustafsson, M.D., Ph.D., Rebecca R. Hung, M.D., Ph.D., Robert L. Gottlieb, M.D., Ph.D., Thibaud Damy, M.D., Ph.D., Alejandra González-Duarte, M.D., Ph.D., Nitasha Sarswat, M.D., Yoshiki Sekijima, M.D., Ph.D., et al., for the APOLLO-B Trial Investigators*

Abstract

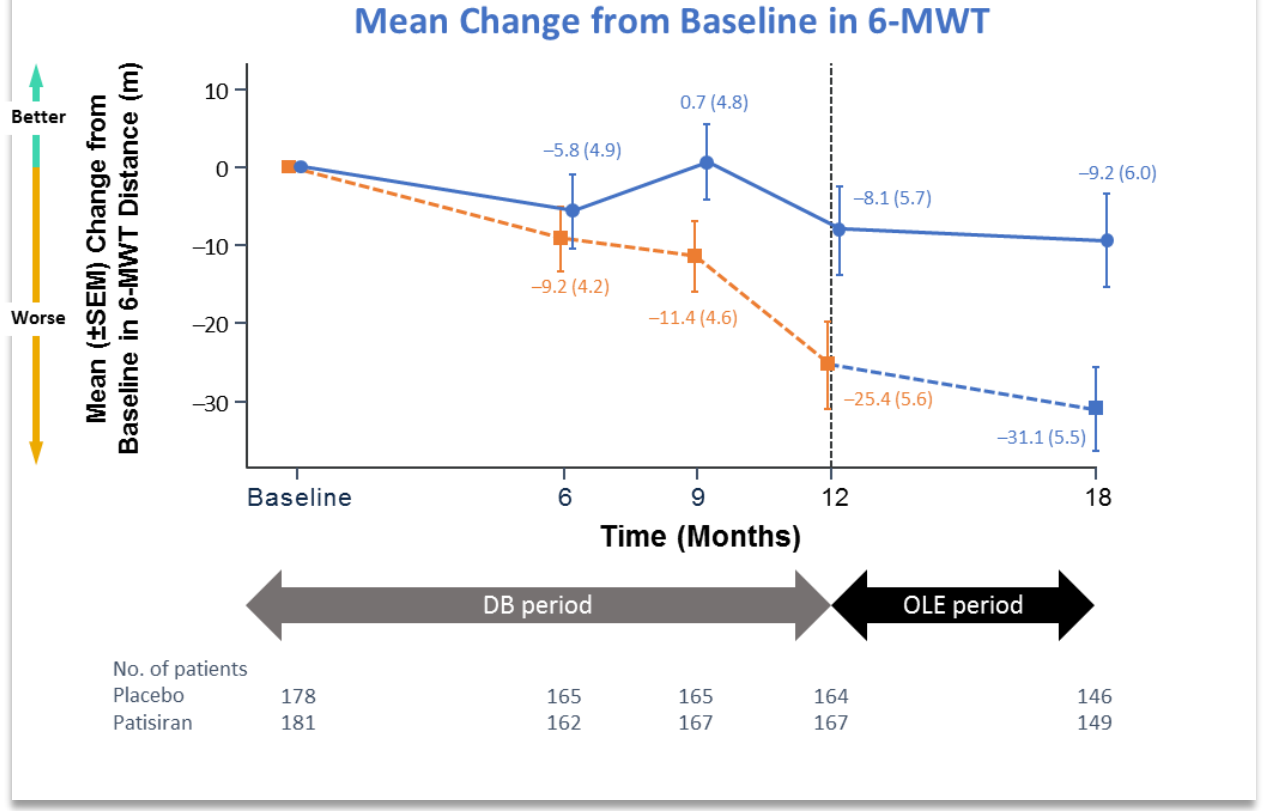
BACKGROUND Transthyretin amyloidosis, also called ATTR amyloidosis, is associated with accumulation of ATTR amyloid deposits in the heart and commonly manifests as progressive cardiomyopathy. Patisiran, an RNA interference therapeutic agent, inhibits the production of hepatic transthyretin.

October 26, 2023
N Engl J Med 2023; 389:1553-1565
DOI: 10.1056/NEJMoa2300757

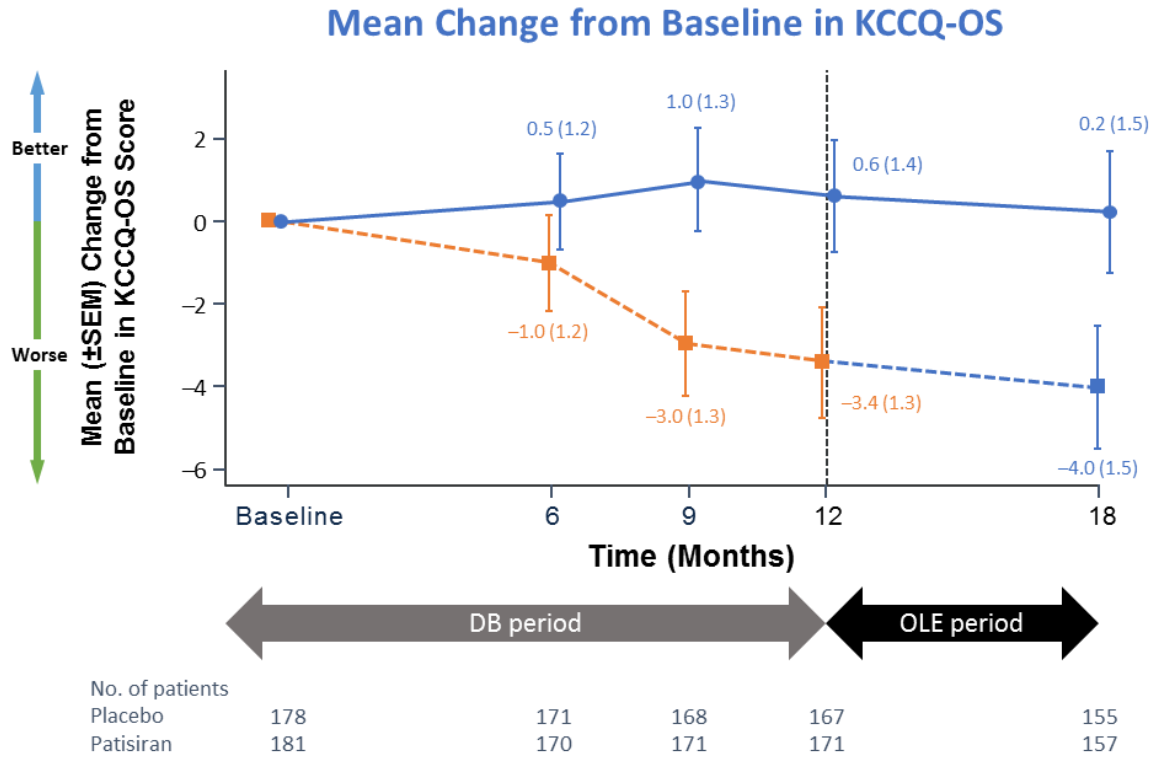
Appolo-B results: ATTR-CA

The NEW ENGLAND JOURNAL of MEDICINE

Primary Endpoint



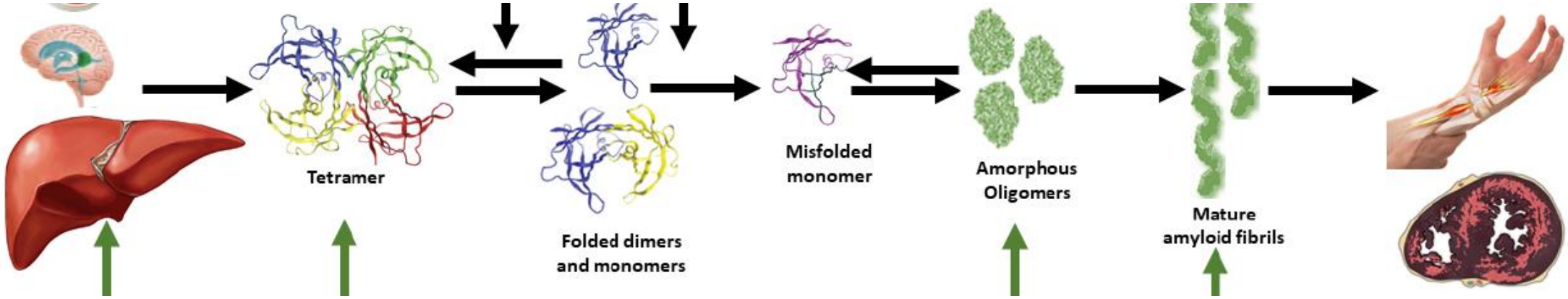
Secondary Endpoint



Patisiran Placebo

Maurer M, NEJM

Mechanisms of action of ATTR-CM new treatments



- Liver Transplantation
- TTR Gene Silencing
  - siRNA and ASO : Patisiran,Revusiran, Vutrisiran, Inotersen, Eplontersen
  - Crispr Cas 9

- TTR Stabilizers
  - Tafamidis,
  - Acoramidis,
  - Diflunisal

- Antibodies to clear Amyloidosis Deposits
  - NI006
  - PRX004

Adapted from Carroll A, et al. J Neurol Neurosurg P 2022

NI006 - Depletion

The NEW ENGLAND JOURNAL of MEDICINE

ORIGINAL ARTICLE

Phase 1 Trial of Antibody NI006 for Depletion of Cardiac Transthyretin Amyloid

Pablo Garcia-Pavia, M.D., Ph.D., Fabian aus dem Siepen, M.D., Erwan Donal, M.D., Ph.D., Olivier Lairez, M.D., Peter van der Meer, M.D., Ph.D., Arnt V. Kristen, M.D., Michele F. Mercuri, M.D., Ph.D., Aubin Michalon, Ph.D., Robert J.A. Frost, M.D., Ph.D., Jan Grimm, Ph.D., Roger M. Nitsch, M.D., Christoph Hock, M.D., et al.

July 20, 2023
N Engl J Med 2023; 389:239-250
DOI: 10.1056/NEJMoa2303765

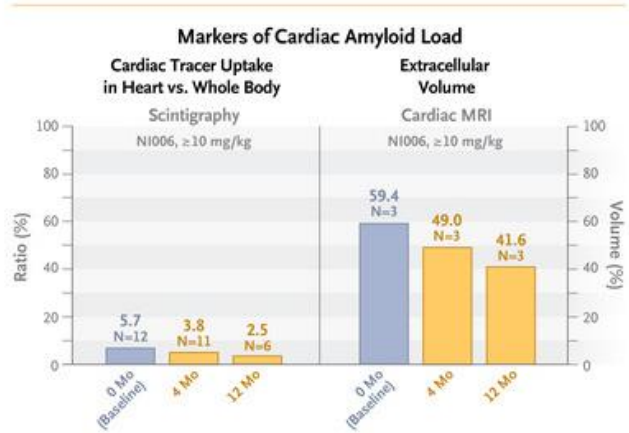
Abstract

BACKGROUND

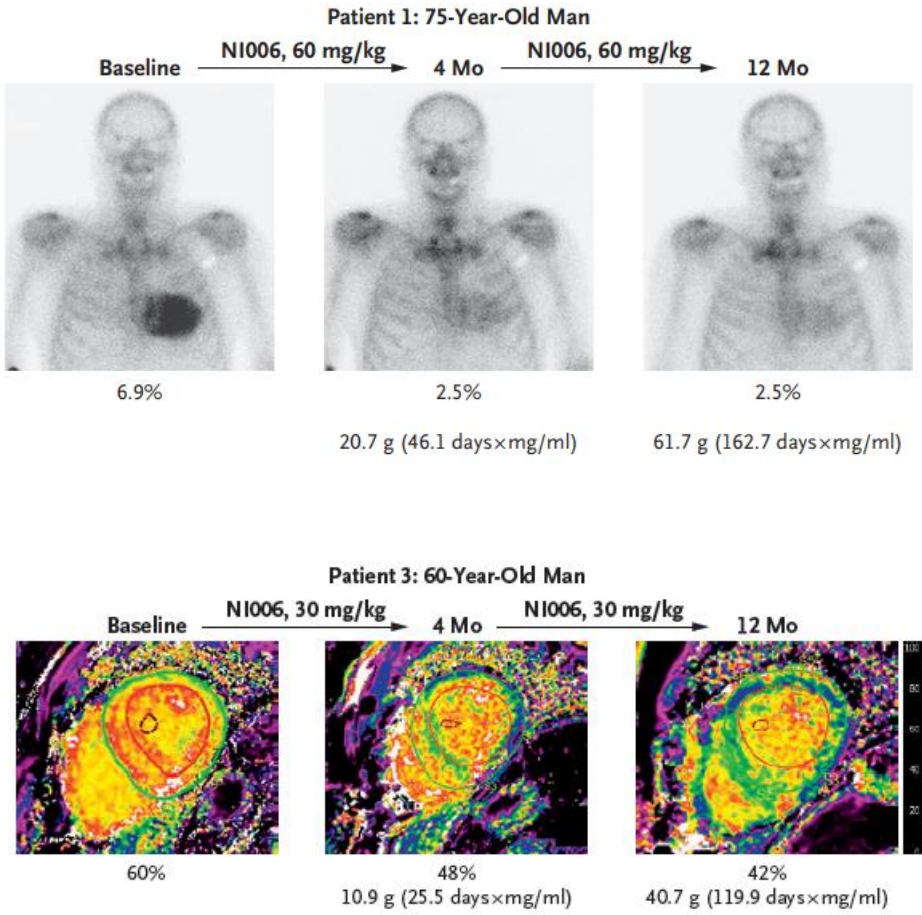
Transthyretin amyloid (ATTR) cardiomyopathy is a progressive and fatal disease caused by misfolded transthyretin. Despite advances in slowing disease progression, there is no available treatment that depletes ATTR from the heart for the amelioration of cardiac dysfunction. NI006 is a recombinant human anti-ATTR antibody that was developed for the removal of ATTR by phagocytic immune cells.

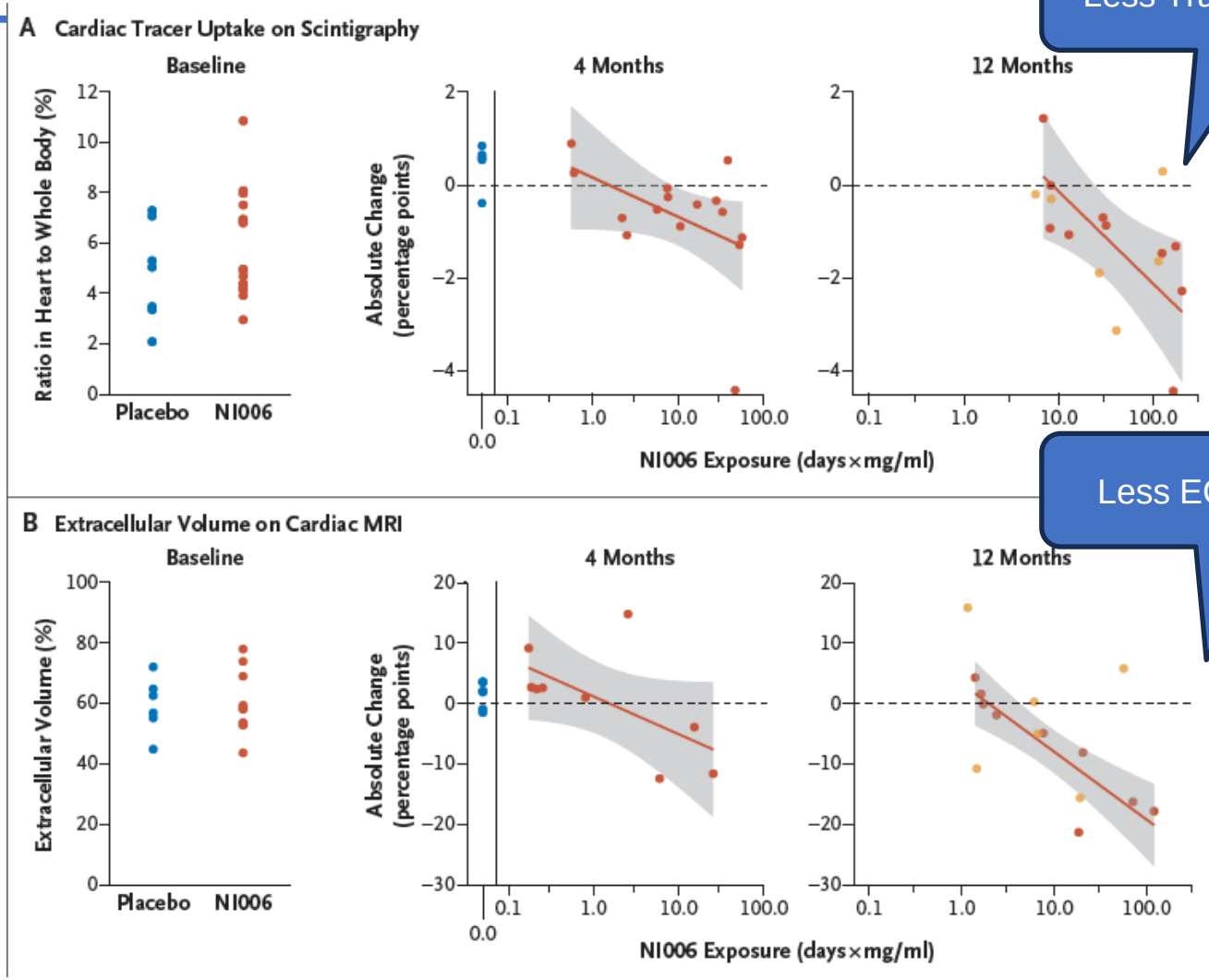
Related Articles

CORRESPONDENCE SEP 28, 2023
Antibody NI006 for Cardiac Transthyretin Amyloid Depletion



Changes in Cardiac Fixation NI006 antibody





Less Tracer

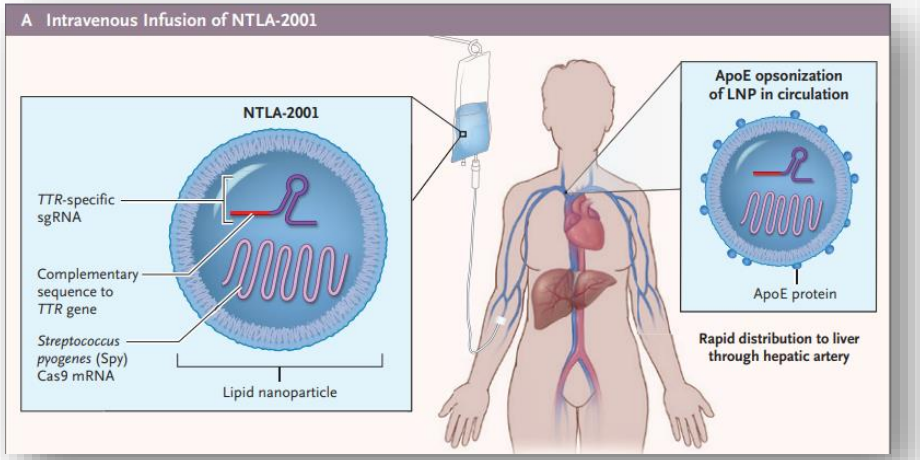
Less ECV

Pablo Garcia-Pavia et al, NEJM 2023, NEJM 2023

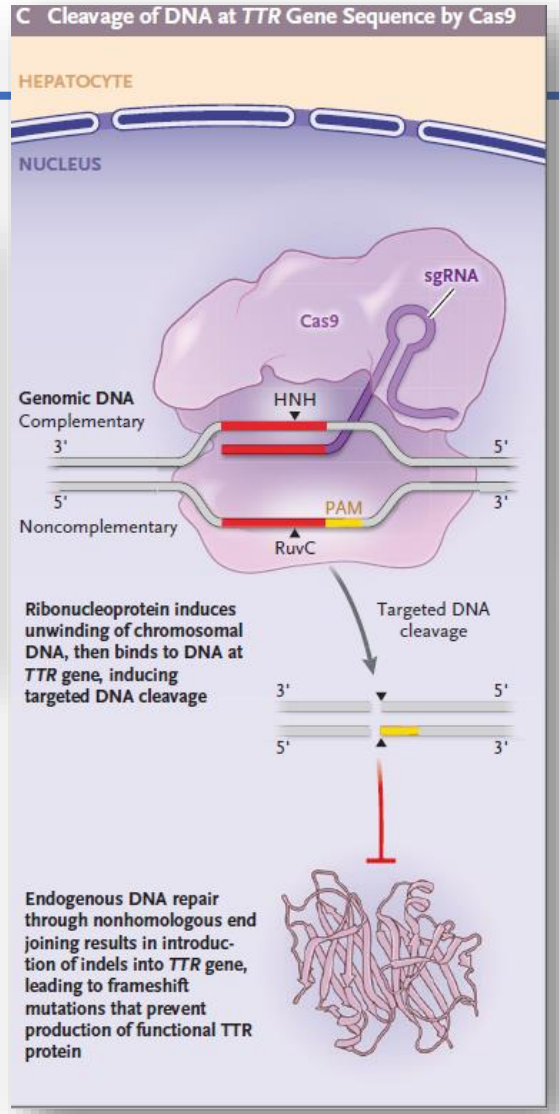
CrisprCas9: First gene therapy results for ATTR : One injection (n=6,

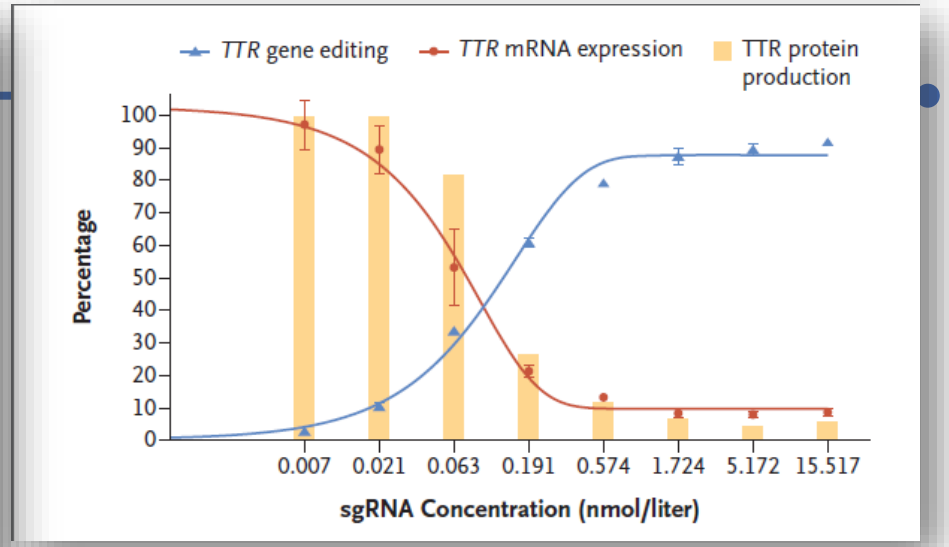
ORIGINAL ARTICLE

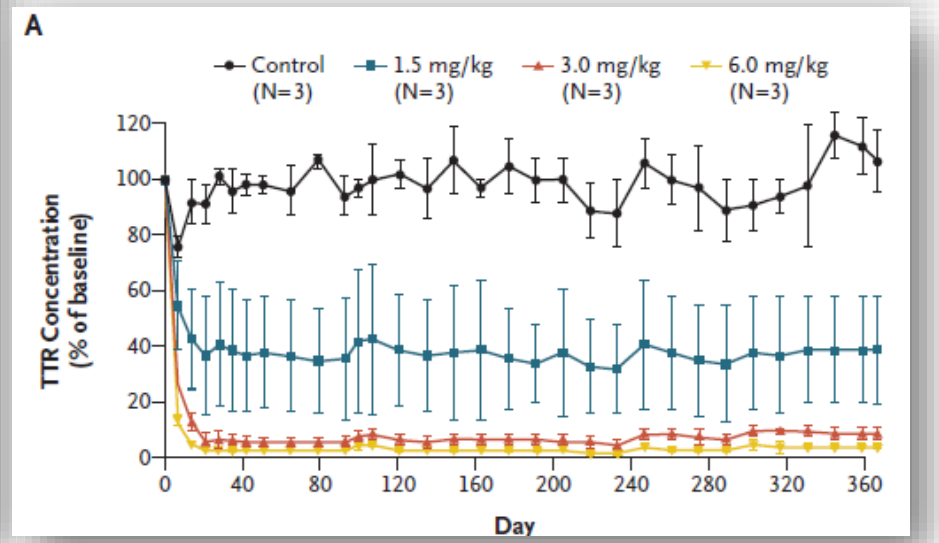
CRISPR-Cas9 In Vivo Gene Editing for Transthyretin Amyloidosis



Clustered regularly interspaced palindromic repeats (CRISPR)/Cas9 is a gene-editing technology makes it possible to correct errors in the genome and turn on or off genes in cells and organisms quickly, cheaply and with relative ease.







Gillmore J et al. NEJM 2021

ECHO AUSTRALIA

The patients

Cardiac Amyloidosis 2025

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