Supplement for:

Durable Vision Improvement After a Single Intravitreal Treatment with Antisense Oligonucleotide in *CEP290*-LCA: Replication in Two Eyes

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Supplemental Figure Legends

Figure S1: RPE melanization evaluated with near-infrared excited autofluorescence imaging. **(Inset, upper left)** A representative near-infrared excited autofluorescence (NIRAF) image of the macular region showing an elliptical region of preserved RPE melanization. Vertical and horizontal intensity profiles through the fovea (gray traces) fit locally with lines (red lines) to determine the extent (black lines). **(A-D)** Change of NIRAF extent from baseline as a function of time from each sepofarsen injection in each eye. Results from superior, inferior, nasal and temporal to the fovea are segregated into different panels. Baseline values for each eye and each panel listed in Table S1. **(E)** Foveal NIRAF intensity used for normalization of the traces. Symbols in all panels comparable to Figures 2 and 3.

Figures S2-S5: Thickness of total retina, ONL, IS and OS layers. **(Inset, upper left)** OCT scan at baseline showing three foveal center locations and two foveal edge locations sampled for retinal layer thicknesses. The longitudinal reflectivity profile (LRP) on the right exemplifies the boundaries used to define the relevant thickness. **(A,B,C)** Thickness change from baseline as a function of time from each sepofarsen injection in each eye. Results from different test locations representing foveal center and foveal edge are segregated into different panels. Each symbol represents measurements from three independent scans. Insets in each panel duplicate the baseline boundaries of NIRAF extent and IS/OS extent shown in Figure 1 as a reference to the locations evaluated (black circles). Symbols in all panels comparable to Figures 2 and 3. Error bars are ±SE. Baseline values for each eye and each panel listed in Table S2.

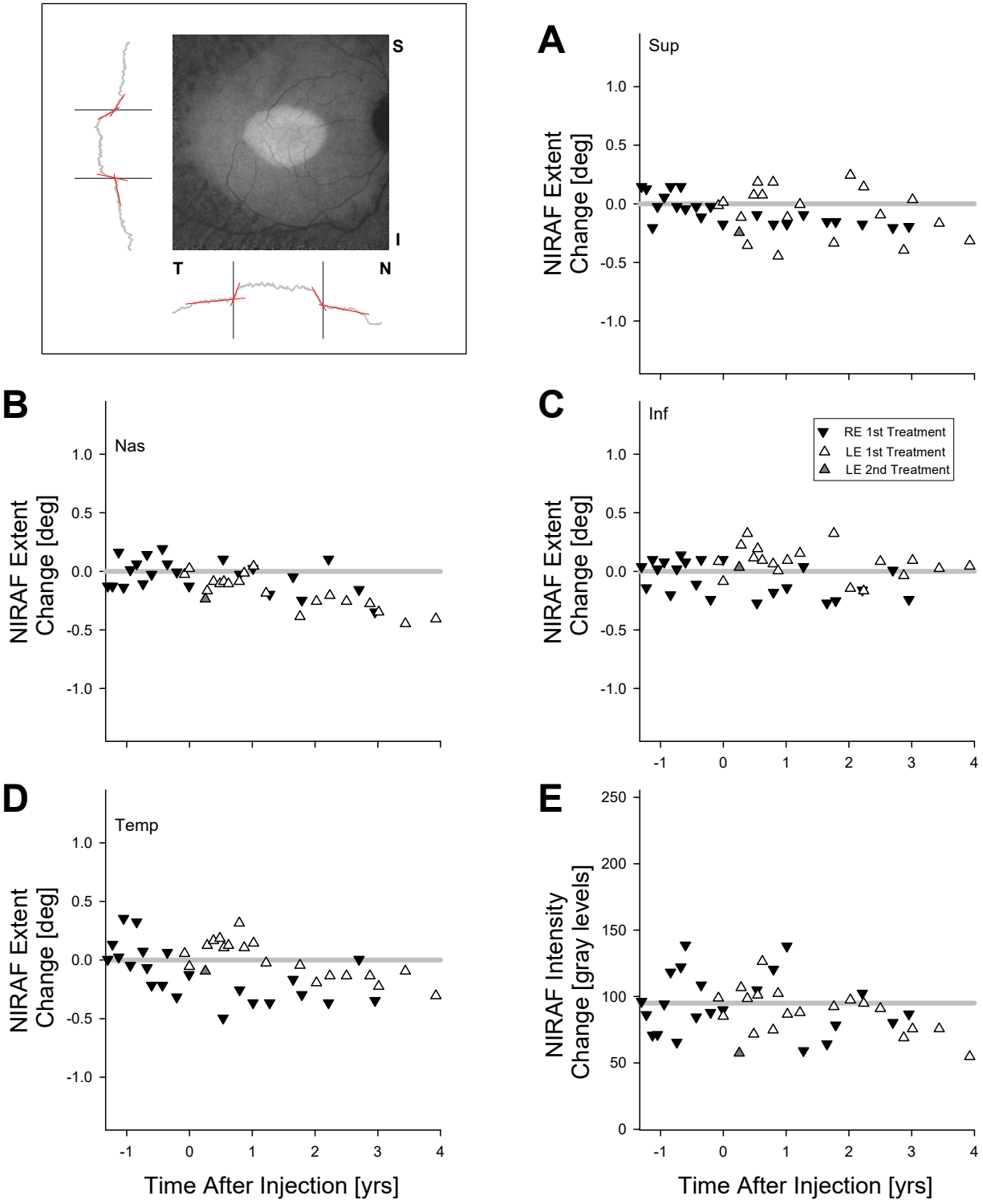
Table S1. NIRAF baseline values used for normalization

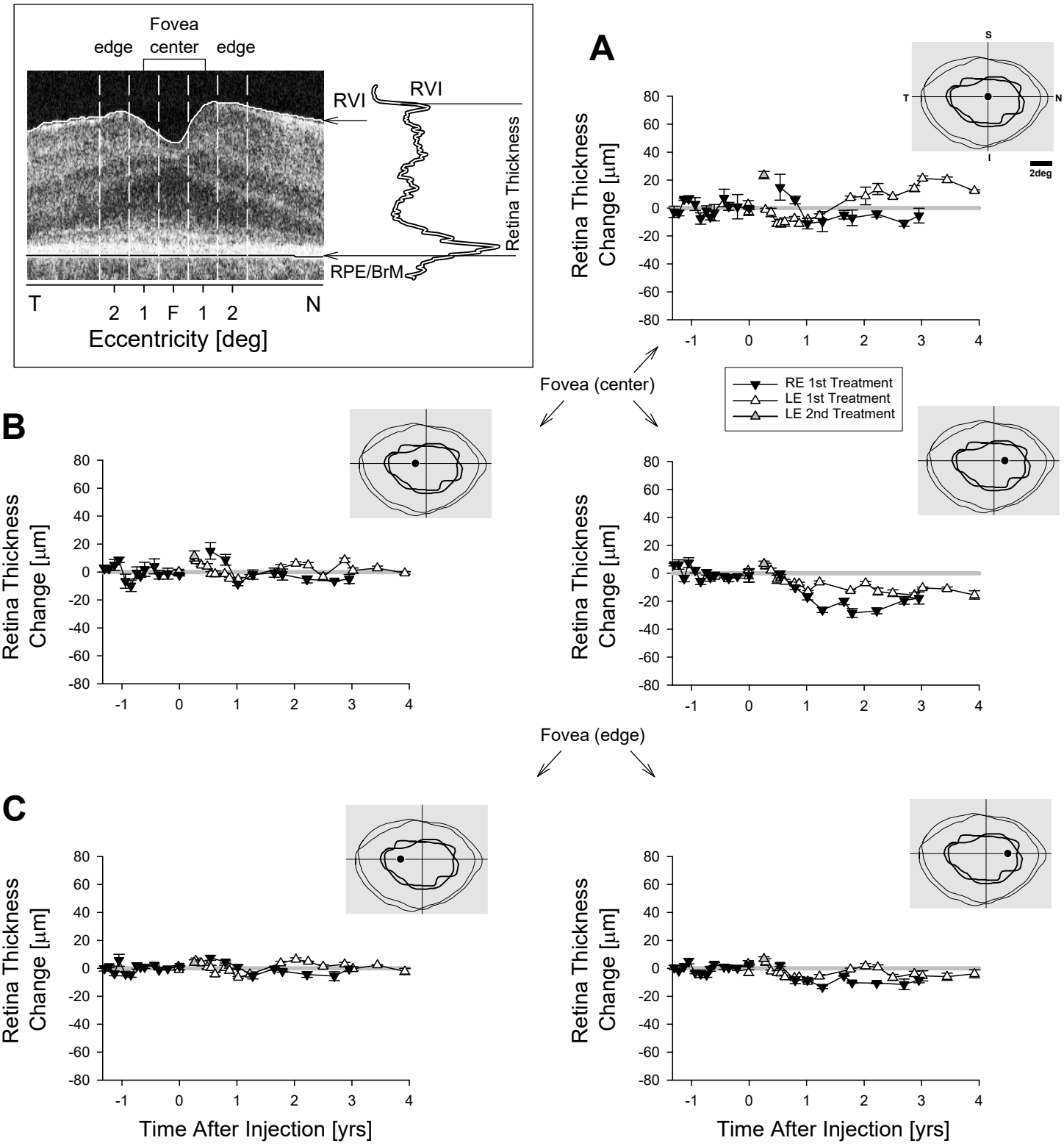
	RE				LE			
	Sup	Inf	Nas	Temp	Sup	Inf	Nas	Temp
Extent [deg]	4.1	5.3	6.5	5.8	4.5	5.0	5.8	6.6

Table S2. OCT baseline values used for normalization

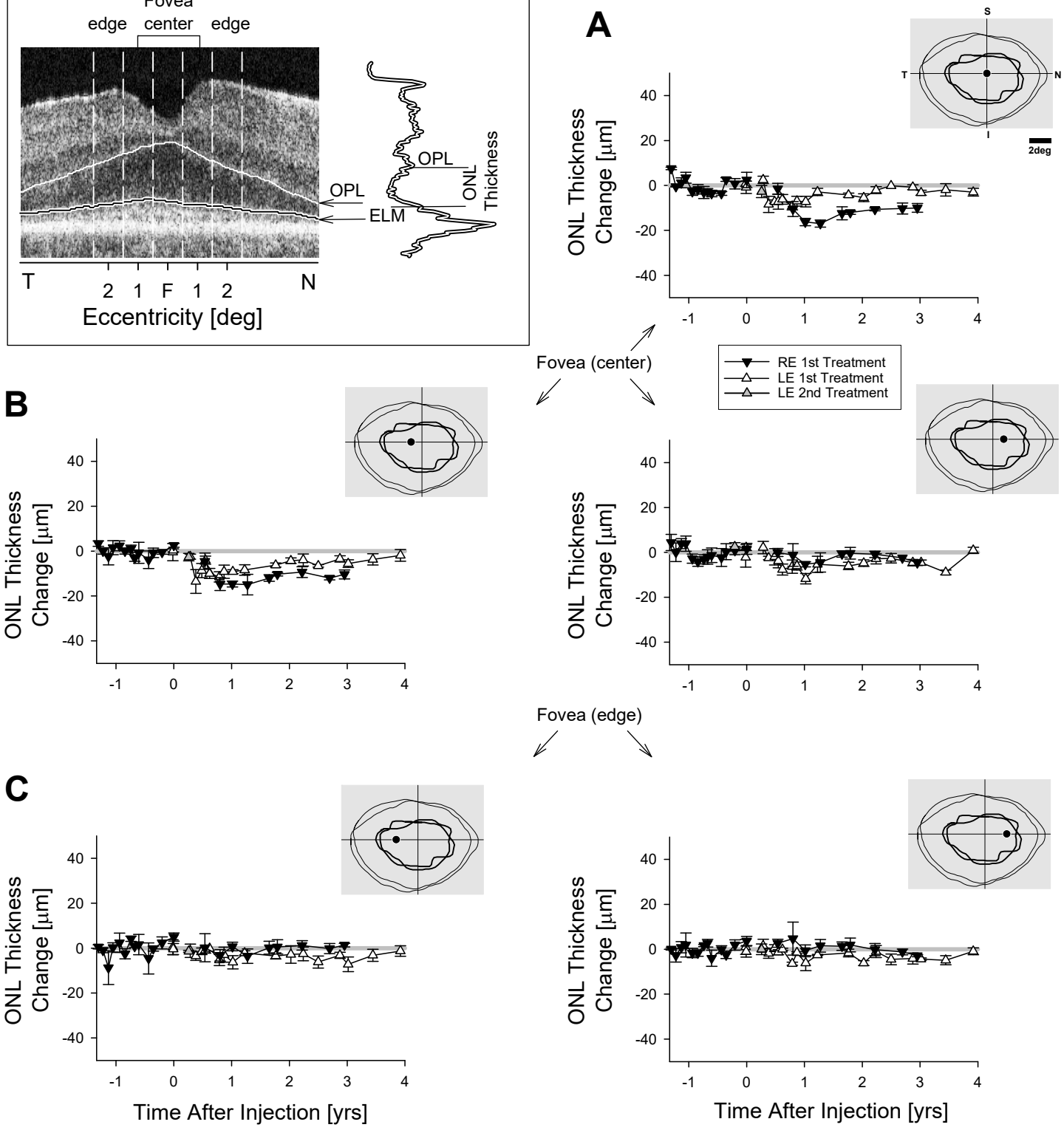
	RE					LE				
	0^o	1^o T	1^o N	2^o T	2^o N	0^o	1^o T	1^o N	2^o T	2^o N
Total retina [μm]	265	297	345	315	343	244	296	324	300	334
ONL [μm]	133	123	111	102	82	123	104	113	84	90
IS [μm]	25	25	24	24	24	24	23	23	21	20
OS [μm]	26	23	21	19	17	27	23	19	17	16
IS/OS [counts]	151	158	66	88	-1	225	163	98	21	-50

S
I
T
N

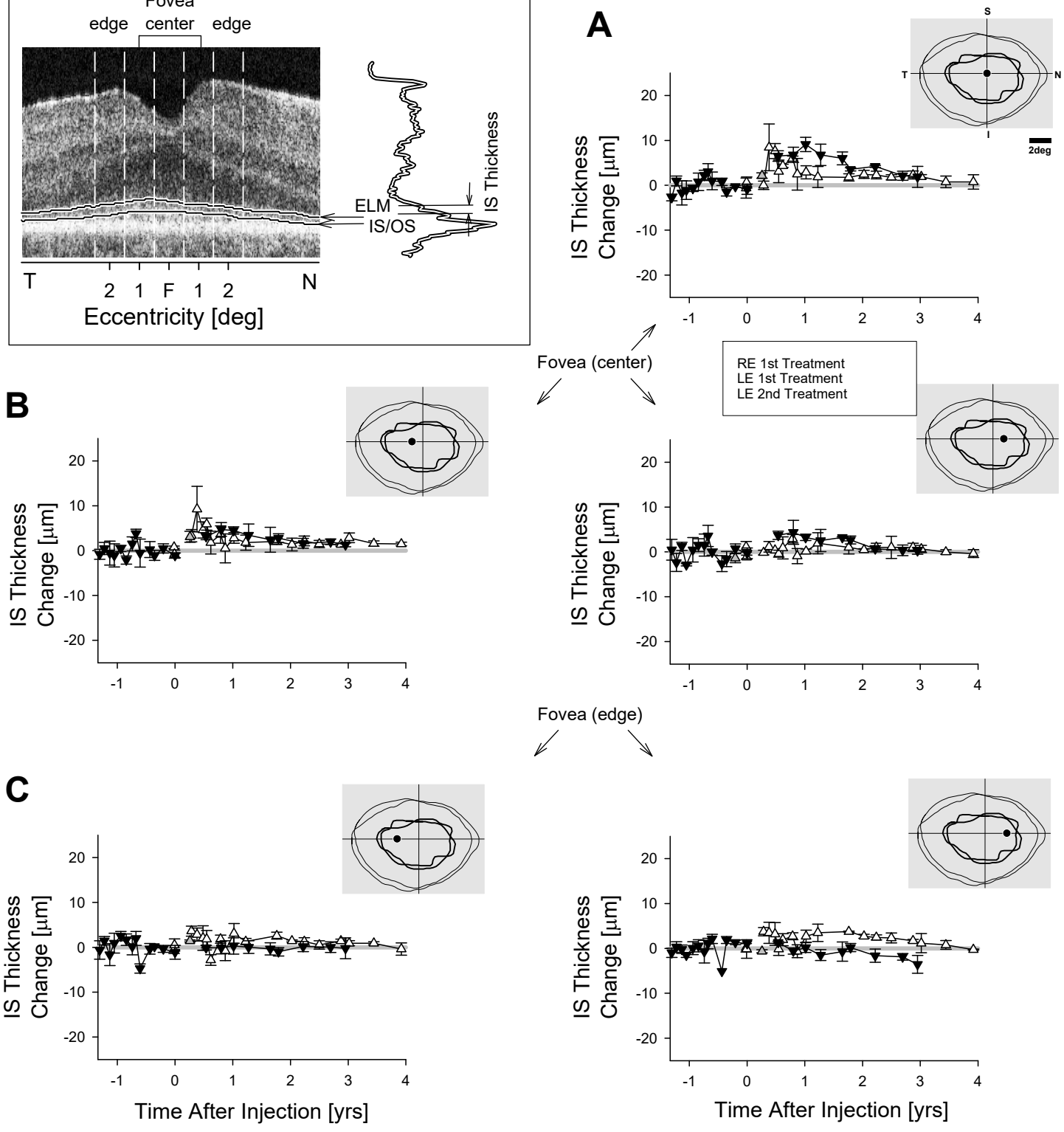

A
Sup
B
Nas
C
Inf
RE 1st Treatment
LE 1st Treatment
LE 2nd Treatment
D
Temp
E
NIRAF Extent Change [deg]
NIRAF Intensity Change [gray levels]
Time After Injection [yrs]


Fovea
edge
center
edge
RVI
RVI
Retina Thickness
RPE/BrM
T
2 1 F 1 2
N
Eccentricity [deg]
A
S
T
N
I
2deg
Retina Thickness Change [µm]
Fovea (center)
RE 1st Treatment
LE 1st Treatment
LE 2nd Treatment
B
Fovea (edge)
C
Time After Injection [yrs]
Time After Injection [yrs]

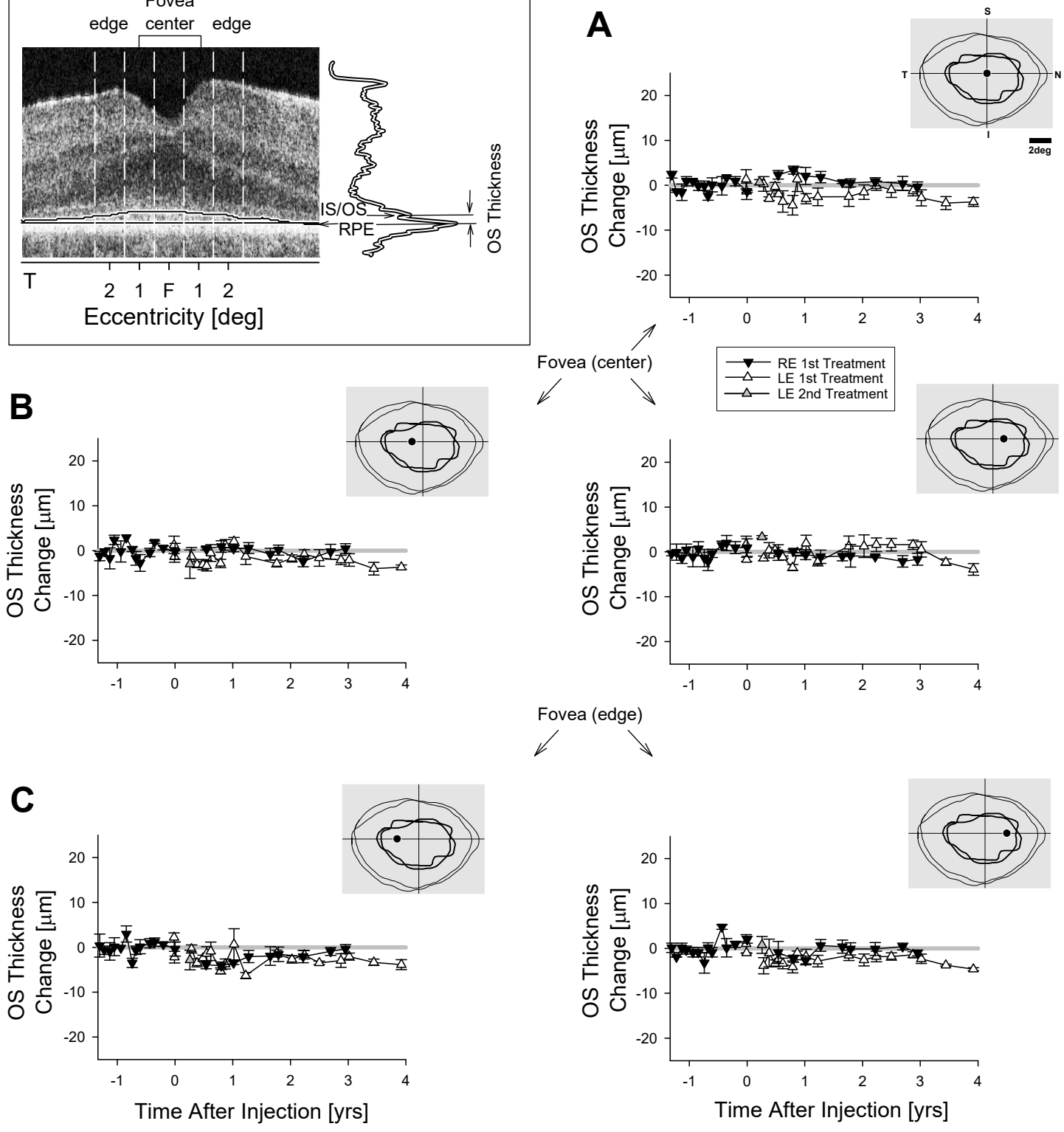
Fovea
edge
center
edge
OPL
ELM
OPL
ONL
Thickness
T
2 1 F 1 2
N
Eccentricity [deg]


A
B
C
S
T
N
I
2deg
ONL Thickness Change [µm]
Fovea (center)
Fovea (edge)
RE 1st Treatment
LE 1st Treatment
LE 2nd Treatment
Time After Injection [yrs]

Fovea
edge
center
edge
ELM
IS/OS
IS Thickness
T
2 1 F 1 2
N
Eccentricity [deg]


A
B
C
S
T
N
I
2deg
IS Thickness Change [µm]
Fovea (center)
Fovea (edge)
RE 1st Treatment
LE 1st Treatment
LE 2nd Treatment
Time After Injection [yrs]

Fovea
edge
center
edge
IS/OS
RPE
OS Thickness
T
2 1 F 1 2
Eccentricity [deg]


A
B
C
S
T
N
I
2deg
OS Thickness Change [µm]
Fovea (center)
Fovea (edge)
RE 1st Treatment
LE 1st Treatment
LE 2nd Treatment
Time After Injection [yrs]