

**XIIIth International
Symposium on Amyloidosis**

**From Misfolded Proteins to
Well-Designed Treatment**



ISA *INTERNATIONAL SOCIETY OF AMYLOIDOSIS*



Groningen Unit for Amyloidosis
Research & Development (GUARD)

May 6 - 10, 2012

Groningen, The Netherlands

University Medical Center Groningen

Program

Sunday, May 6

- 12.00 Registration opens in UMCG (*'Fonteinpatio'*)
- 15.30-16.00 Coffee**
- 16.00-16.30 Opening ceremony ('Blauwe Zaal')**
- 16.00-16.10 Welcome address - Van Rijswijk
- 16.10-16.20 Introduction to the symposium - Hazenberg
- 16.20-16.30 A patient's experience and perspective - Magalhaes
- 16.30-17.00 Opening Lecture: History of Amyloidosis - Kyle**
- 17.00-17.30 Keynote Lecture: Amyloid in semen boosts HIV-1 transmission - Münch**
- 18.00-18.30 Walk to the University Building
- 18.30-20.30 Welcome party;** offered to you by the University of Groningen, the Municipality of Groningen and the Province of Groningen (*University Building*)
- 18.30-18.45 Welcome address - Poppema
- 18.45-20.30 Refreshments, party
- 20.00-21.30 ISA Board Meeting (*'Engelse Zaal' in University Building*)

Monday, May 7

- 7.30-8.00 Coffee ('Fonteinpatio')**
- 8.00-8.30 Enno Mandema Memorial Lecture ('Blauwe Zaal'):**
Amyloidosis: 1979-2012-... - Sir Mark Pepys
- 8.30-10.20 Plenary session 1: Fibril and Amyloid Formation.** Chairmen: Seldin and Limburg
- 8.35 State of the art - Fändrich
- 8.50 OP 01 Aggregates of the Alzheimer's Disease Amyloid Peptide A β 40 Studied by Solid State NMR. Lopez del Amo (München, Germany)
- 9.02 OP 02 Structural modulation of A β fibrils by glycosaminoglycans. Madine (liverpool, UK)
- 9.14 OP 03 Structure and pathogenicity of mature fibrils and their structural precursors. Fändrich (Halle, Germany)
- 9.26 OP 04 Towards a cure for Parkinson's Disease: inhibitors of α -synuclein aggregation. Lorenzen (Aarhus, Denmark)
- 9.38 OP 05 In vivo and in vitro anti-Amyloid functions of the systemic amyloid precursor Transthyretin (TTR). Buxbaum (San Diego, USA)
- 9.50 OP 06 Two types of fibril compositions in ATTR amyloidosis and their correlation to clinical phenotype. Ihse (Uppsala, Sweden)
- 10.02 Perspectives - Bellotti
- 10.20-10.45 Coffee break ('Fonteinpatio')**
- 10.45-12.30 Plenary session 2: Cell and tissue targeting and toxicity ('Blauwe Zaal')**
Chairmen: Grateau and Luiten
- 10.45 State of the art - Kisilevsky
- 11.00 OP 07 Biochemical properties of highly virulent prion strains. Sigurdson (San Diego, USA)
- 11.12 OP 08 Detection of endogenous amyloid fibrils in human semen. Usmani (Ulm, Germany)

- 11.24 OP 09 A proteomic approach to unravel the cytotoxic responses to different aggregation-prone proteins. Picotti (Zürich, Switzerland)
- 11.36 OP 10 Investigating vulnerability to proteasome inhibition in primary light chain amyloidosis
Oliva (Milano, Italy)
- 11.48 OP 11 Unique features that characterize urinary exosomes in light chain amyloidosis (AL).
Ramirez-Alvarado (Rochester, USA)
- 12.00 OP 12 Fighting familial amyloidosis using an impaired ER-stress response yeast strain.
Rodrigues (Lisboa, Portugal)
- 12.12 Perspectives - Buxbaum
- 12.30-14.00 Lunch** (Sponsor Spectral Imaging) in the '*Fonteinpatio*'
- 13.00-14.00 Poster viewing** individually ('*Blauwe Patio*')
- 14.00-16.00 Plenary session 3: Animal models and cell culture systems** ('*Blauwe Zaal*')
Chairpersons: Gruys and G. Westermark
- 14.00 State of the art - Saraiva
- 14.15 OP 13 Heparin/Chitosan nanoparticle delivery system for in vivo evaluation of glycosaminoglycans influence on transthyretin deposition. Gonçalves (Porto, Portugal)
- 14.27 OP 14 Multi-system disease-specific induced pluripotent stem cell modeling of familial amyloidosis. Leung (Boston, USA)
- 14.39 OP 15 Anti-amyloid drug development using *Drosophila* as a model. Segal (Tel Aviv, Israel)
- 14.51 OP 16 *C. elegans* expressing human β 2-m recapitulates the molecular mechanisms underlying dialysis-related amyloidosis. Soria (Pavia, Italy)
- 15.03 OP 17 Doxycycline Interaction with Amyloidogenic Proteins and Activity in Mouse Models.
Ward (Boston, USA)
- 15.15 OP 18 An all human cell culture model of AA amyloid formation: human serum amyloid A (SAA) and human peripheral blood mononuclear cells (PBMC). Ishii (Indianapolis, USA)
- 15.27 OP 19 Macrophage-mediated natural clearance of Amyloid A is not impaired in the absence of immunoglobulins or central complement factors. Sponarova (Zürich, Switzerland)
- 15.40 Perspectives - Kluve-Beckerman
- 16.00-16.30 Tea break** ('*Fonteinpatio*')
- 16.30-17.30 Poster viewing** (PA 1-65) in 5 groups ('*Blauwe Patio*')
- 17.30-18.00 Selected poster presentations** PA 15, PA 22, PA 51, PA 53, and PA 54 ('*Blauwe Zaal*')
Chairmen: Limburg and Gruys
- 17.00-18.00 Meetings: Nomenclature Committee; other working groups and committees ('*Lokaal 10, 15*')
- 18.00-19.30 Buffet in UMCG** ('*Fonteinpatio*')
- 19.30-22.00 Plenary session 4: Looking for consensus** ('*Blauwe Zaal*')
- 19.30 Early detection of amyloid; reporting - Picken, P. Westermark, Hazenberg
- 20.20 Organ involvement and response criteria in AL - Gertz, Wechalekar, Palladini
- 21.10 Organ involvement and response criteria in non-AL - Suhr, Obici, Lachmann, Merckies
- 22.00 Closing
- Tuesday, May 8**
- 7.30-8.00 Coffee** ('*Fonteinpatio*')
- 8.00-10.05 Plenary session 5: Diagnosis and typing: Histochemistry and proteomics** ('*Blauwe Zaal*')
Chairmen: Linke and Kluin

- 8.00 State of the art - Röcken
- 8.15 OP 20 Amyloid fibrils possess characteristic electronegative fingerprints that can be distinguished by poly-basic peptides. Wall (Knoxville, USA)
- 8.27 OP 21 Luminescent conjugated oligothiophenes: two new dyes for amyloid screening diagnostics. Sjölander (Linköping, Sweden)
- 8.39 OP 22 Accuracy of routine amyloid typing using immunohistochemistry on a large number of consecutive patients: preconditions for its success and validation. Linke (Martinsried, Germany)
- 8.51 OP 23 An indirect ELISA for transthyretin quantification in fat tissue of patients with ATTR Amyloidosis. Hazenberg (Groningen, The Netherlands)
- 9.03 OP 24 A native human monoclonal IgG with pan-amyloid binding specificity. Dessain (Wynnewood, USA)
- 9.15 OP 25 Diagnosis of Amyloidosis Subtype by Laser-Capture Microdissection (LCM) and Tandem Mass Spectrometry (MS) Proteomic Analysis. Mollee (Brisbane, Australia)
- 9.27 OP 26 Mass spectrometry based proteomics for classification of amyloidosis: Mayo Clinic Experience. Dogan (Rochester, USA)
- 9.39 OP 27 A comparison of immunohistochemistry and mass spectrometry for determining the amyloid fibril protein from formalin fixed biopsy tissue. Gilbertson (London, UK)
- 9.51 Perspectives - Dogan
- 10.05-10.30 Coffee break ('Fonteinpatio')**
- 10.30-12.30 Plenary session 6: Imaging in amyloidosis ('Blauwe Zaal')**
Chairmen: Van Rijswijk and Glaudemans
- 10.30 State of the art - Hawkins
- 10.45 OP 28 Patterns of late gadolinium enhancement and survival in cardiac amyloidosis: a systematic review of 95 patients with AL or ATTR type. Dungu (London, UK)
- 10.57 OP 29 ^{99m}Tc-3,3-Diphosphono-1,2-Propanodicarboxylic Acid (^{99m}Tc DPD) scintigraphy in 171 patients with suspected systemic amyloidosis. Hutt (London, UK)
- 11.09 OP 30 Accuracy of ^{99m}Tc-HMPD myocardial scintigraphy for the diagnosis of cardiac involvement in patients with familial amyloid polyneuropathie. Algalarrondo (Clamart, France)
- 11.21 OP 31 Iodine-123 metaiodobenzylguanidine for the evaluation of cardiac sympathetic denervation in early stage amyloidosis. Noordzij (Groningen, The Netherlands)
- 11.33 OP 32 The added value of SPECT(-CT) compared with planar ¹²³I-SAP scintigraphy in patients with systemic amyloidosis. Van Rheenen (Groningen, The Netherlands)
- 11.45 OP 33 AL amyloid imaging and therapy with an amyloid specific monoclonal antibody. Wall (Knoxville, USA)
- 11.57 OP 34 Radioimmunoimaging of Patients with AL Amyloidosis. Wells (Knoxville, USA)
- 12.10 Perspectives - Wall
- 12.30-14.00 Lunch (Sponsor Alnylam) in the 'Fonteinpatio'**
- 13.00-14.00 Poster viewing individually ('Blauwe Patio')**
- 14.00-16.30 Plenary session 7: Biology, clinics and prognosis in AL amyloidosis ('Blauwe Zaal')**
Chairpersons: Comenzo and Croockewit
- 14.00 State of the art - Merlini

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- 14.15 OP 35 Diagnostic performance of the novel monoclonal assay for the measurement of circulating free light chain (FLC) in 220 consecutive newly-diagnosed patients with AL amyloidosis. Palladini (Pavia, Italy)
- 14.27 OP 36 A Revised Prognostic Staging System for Light Chain Amyloidosis (AL) Incorporating Cardiac Biomarkers and Serum Free Light Chain Measurements. Kumar (Rochester, USA)
- 14.39 OP 37 Coronary Microvascular Function in Cardiac Amyloidosis. Dorbala (Boston, USA)
- 14.51 OP 38 Depressed midwall fractional shortening is a powerful prognostic determinant in cardiac AL amyloidosis. Perlini (Pavia, Italy)
- 15.03 OP 39 ALchemy - A Large Prospective 'Real World' Study of Chemotherapy in AL Amyloidosis. Gillmore (London, UK)
- 15.15 OP 40 Prognostic significance of cytogenetic aberrations in light chain amyloidosis patients treated with melphalan / dexamethasone as first-line therapy. Schönland (Heidelberg, Germany)
- 15.27 OP 41 A Framework for Clinical Research in Systemic Light-chain (AL) Amyloidosis: Consensus Report of the First Amyloidosis Foundation Roundtable. Comenzo (Boston, USA)
- 15.39 OP 42 An Italian single center prospective study on outcomes in AL amyloidosis. Palladini (Pavia, Italy)
- 15.51 OP 43 Outcomes and Treatment of Relapsed Systemic Amyloidosis. Warsame (Rochester, USA)
- 16.03 OP 44 Solid organ transplantation in AL amyloidosis and monoclonal immunoglobulin deposition disease: the French experience. Desport (Poitiers, France)
- 16.15 Perspectives - Gertz

- 14.00 Welcome and Aim of the Workshop - Hammarström
- 14.30 The use of LCP probes for classification of amyloid deposits - Nilsson
- 15.30 Hyperspectral-imaging for direct analysis of LCP-coupled amyloid deposits - Sluszny
- 16.30-17.00 Tea break ('Fonteinpatio')**

- Chair: Hazenberg
- 17.00 Welcome - Hazenberg
- 17.05 Unravelling the TTR misfolding hypothesis - Kelly
- 17.30 New approaches to the management of TTR-FAP – Coelho
- 17.55 Conclusions - Hazenberg
- 18.00-19.30 Drinks and snacks** (Sponsor Proteotech) in the 'Fonteinpatio'
- 18.00-19.00 Poster viewing** (PB 1-66) in 5 groups ('Blauwe Patio')
- 19.00-19.30 Selected poster presentations** PB 12, PB 41, PB 45, PB 47, and PB 66 ('Blauwe Zaal')
- Chairpersons: Croockewit and Van Rijswijk
- 18.30-19.30 Committee Meetings ('Lokaal 15 and W2270')
- 19.30-20.00 Walk to the restaurant
- 20.00-22.30 Dinner in Ni Hao wok restaurant**

Wednesday, May 9

- 7.30-8.00** **Coffee** (*'Fonteinpatio'*)
- 8.00-9.50** **Plenary session 8: Biology, clinics and prognosis in ATTR amyloidosis** (*'Blauwe Zaal'*)
Chairmen: Suhr and Van den Berg
- 8.00 State of the art - Ikeda
- 8.15 OP 45 First online registry of mutations in hereditary amyloidosis: amyloidosismutations.com.
Rowczenio (London, UK)
- 8.27 OP 46 The Prevalence of Holter Abnormalities in ATTR Cardiac Amyloidosis. Maurer (New
York, USA)
- 8.39 OP 47 Circadian rhythm of blood pressure reflects the severity of cardiac impairment
in Familial Amyloid Polyneuropathy. Algalarrondo (Clamart, France)
- 8.51 OP 48 Senile Systemic Amyloidosis: a large cohort study detailing clinical features, laboratory
results, and survival. Connors (Boston, USA)
- 9.03 OP 49 Systematic review of 1142 admissions with acute heart failure reveals high frequency
of transthyretin V122I cardiac amyloidosis in Afro-Caribbean patients. Dungu (London,
UK)
- 9.15 OP 50 Comparison of V122I and Senile Cardiac Amyloidosis: Differences in Clinical Features
and Outcomes. Maurer (New York, USA)
- 9.30 Perspectives - Berk
- 9.50-11.05** **Plenary session 9: Biology, clinics and prognosis in AA (and other types of) amyloidosis**
Chairpersons: Lachmann and Van Gameren
- 9.50 State of the art - Livneh
- 10.05 OP 51 A single centre 20 year case series of AA amyloidosis – changing epidemiology.
Lachmann (London, UK)
- 10.17 OP 52 Long term effectiveness of surgery in localized laryngeal amyloidosis. Hazenberg
(Groningen, The Netherlands)
- 10.29 OP 53 First liver and kidney transplant for leukocyte chemotactic factor 2-amyloidosis
presenting with acute liver failure. Fix (San Francisco, USA)
- 10.41 OP 54 The role of liver transplantation in the hereditary amyloidoses; the U.K experience.
Stangou (Birmingham, UK)
- 10.53 Perspectives - Skinner
- 11.05-11.30** **Coffee break** (Sponsor GSK) in the *'Fonteinpatio'*
- 11.30-13.00** **Plenary session 10: Therapy of ATTR amyloidosis** (*'Blauwe Zaal'*)
Chairpersons: Haagsma and Kuks
- 11.30 State of the art - Ando
- 11.45 OP 55 Familial Dynamics, Attachment and Psychopathology in FAP Patients. Lopes (Porto,
Portugal)
- 11.57 OP 56 Tolerability of diflunisal therapy in patients with transthyretin amyloidosis. Whelan
(London, UK)
- 12.09 OP 57 Can development of post liver transplant cardiomyopathy be predicted from amyloid
fibril composition? Suhr (Umea, Sweden)
- 12.21 OP 58 Long term Effect of Liver transplantation on FAP on the neuropathy: Risk factors for
progression of the walking disability. The 18 years French experience: a monocentric
study in 200 patients. Adams (Paris, France)

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- 12.33 OP 59 Treatment of Advanced Heart Failure in Cardiac Amyloidosis with Left Ventricular Assist Device Therapy. Swiecicki (Rochester, USA)
- 12.45 Perspectives - Zeldenrust
- 13.00-14.30 Lunch ('Fonteinpatio')**
- 13.30-14.30 Poster viewing** individually ('Blauwe Patio')
- 13.00-14.30 Business meeting of the ISA ('Lokaal 16')
- 14.30-16.15 Plenary session 11: Chemotherapy in AL amyloidosis ('Blauwe Zaal')**
Chairmen: Palladini and Vellenga
- 14.30 State of the art - Dispenzieri
- 14.45 OP 60 Evaluation of an early switch to second line chemotherapy in AL amyloidosis among patients who fail to achieve a very good partial response to frontline treatment. Wechalekar (London, UK)
- 14.57 OP 61 A phase I / II study of Lenalidomide with low dose oral cyclophosphamide and low dose dexamethasone (RdC) in AL amyloidosis. Kastritis (Athens, Greece)
- 15.09 OP 62 Efficacy of Bortezomib/Cyclophosphamide/Dexamethasone (VCD) Chemotherapy In Naive Patients with High Risk Cardiac Light Chain Amyloidosis (Mayo Clinic stage III). Jaccard (Limoges, France)
- 15.21 OP 63 Salvage therapy with bendamustine and prednisone (BeP) in AL amyloidosis: a pilot study. Milani (Pavia, Italy)
- 15.33 OP 64 The Activity of Pomalidomide in Patients with Immunoglobulin Light Chain Amyloidosis. Dispenzieri (Rochester, USA)
- 15.45 OP 65 Phase I study of MLN9708, a novel, investigational oral proteasome inhibitor, in patients with relapsed or refractory light-chain amyloidosis (AL). Sanchorawala (Boston, USA)
- 16.00 Perspectives - Schönland
- 16.15-16.30 ISA information: AMYLOID Journal – P. Westermark**
- 16.30-17.00 Tea break** (Sponsor GSK) in the 'Fonteinpatio'
- 16.30-17.15 Poster viewing** (PC 1-66) in 5 groups ('Blauwe Patio')
- 17.15-17.45 Selected Poster presentations** PC 08, PC 28, PC 45, PC 50, and PC 61 ('Blauwe Zaal')
Chairpersons: Minnema and Kuks
- 17.45-18.00 Walk to the Groninger Museum
- 18.00-20.00 Visit Groninger Museum**
- 20.00-20.30 Walk to the Der Aa Church
- 20.30-23.30 Congress Dinner and awards presentation ('Der Aa Church')**

Thursday, May 10

- 7.30-8.00 Coffee** ('Fonteinpatio')
- 8.00-10.30 Plenary session 12: Design of targeted molecules and innovative drugs ('Blauwe Zaal')**
Chairmen: Ando and Hazenberg
- 8.00 State of the art - Seldin
- 8.15 OP 66 Intrinsic Apoptosis Can Occur Promptly After Silencing Lambda Light Chain Genes in Human Clonal Plasma Cells. Zhou (Boston, USA)

- 8.27 OP 67 Marked Removal and Clearance of AA Amyloid Deposits in Target Organs (Kidney, Liver and Spleen) by the Small Molecule Systebryl™ Following Oral Administration: A Potential Break-through Drug for the Treatment of Systemic AA Amyloidosis. Snow (Kirkland, USA)
- 8.39 OP 68 A clinical Phase 3 confirmatory trial of eprodisate in the treatment of AA amyloidosis patients. Garceau (Laval, Canada)
- 8.51 OP 69 The Diflunisal Trial: Demographics, baseline neurologic staging, and adverse events. Berk (Boston, USA)
- 9.03 OP 70 Curcumin as a novel natural compound acting as TTR amyloidosis inhibitor *in vivo*. Ferreira (Porto, Portugal)
- 9.15 OP 71 Safety and efficacy of doxycycline plus tauroursodeoxycholic acid in transthyretin amyloidosis. Obici (Pavia, Italy)
- 9.27 OP 72 Antibody therapy against amyloid forms of transthyretin for familial amyloidotic polyneuropathy. Su (Kumamoto, Japan)
- 9.39 OP 73 Clinical Development of an Antisense Therapy for the Treatment of Hereditary Transthyretin Amyloidosis. Ackermann (Carlsbad, USA)
- 9.51 OP 74 Final Phase I safety, pharmacokinetic and pharmacodynamic results for ALN-TTR01, a novel RNAi therapeutic for the treatment of transthyretin amyloidosis. Coelho (Porto, Portugal)
- 10.03 OP 75 RNAi therapy using cholesterol-conjugated siRNA for TTR-related ocular amyloidosis. Tasaki (Kumamoto, Japan)
- 10.15 Perspectives - Benson
- 10.30-11.00 Coffee break ('Fonteinpatio')**
- 11.00-12.00 Closing session: Conclusions and prospects ('Blauwe Zaal')**
Chair: Van Rijswijk
- 11.00 Monday: Basic research - P. Westermark
- 11.15 Tuesday/Wednesday: Clinical challenges - Skinner
- 11.30 Wednesday/Thursday: Therapeutic prospects - Merlini
- 11.45 Closing remarks - Hazenberg
- 12.00-13.00 Lunch ('Fonteinpatio')**
- 12.00-13.00 Certificates of attendance ('Fonteinpatio')
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- 14.00-18.00 PATIENTS' AFTERNOON: LIVING WITH AMYLOID**
- 13.30-14.00 Registration and coffee ('Fonteinpatio')**
- 14.00-15.20 Plenary session 1: Information about Amyloidosis, the disease ('Blauwe Zaal')**
Chair: Hazenberg
- 14.00 Welcome, introduction - Hazenberg
- 14.05 Amyloid, typing, disease manifestations - Hazenberg
- 14.20 Neuropathy as example of ATTR amyloidosis; treatment principles - Vrancken
- 14.35 Cardiomyopathy and nephropathy as examples of AL: treatment principles - Minnema
- 14.50 New developments in diagnosis; an overview - Skinner
- 15.05 New developments in treatment; an overview - Merlini

Program

- 15.20-15.40** **Tea break, collection of questions** (*'Fonteinpatio'*)
- 15.40-17.00** **Plenary session 2: Do we need a patients' organization?** (*'Blauwe Zaal'*)
- 15.40 Amyloidosis in Australia - Neely
- 15.50 Participation of patients and their organizations in research, cure and care - Oosterwijk
- 16.05 My experiences in establishing a patient's organization for a rare disease - Smit
- 16.20 Panel discussion
- 16.50 Summary of the afternoon; prospects and plans; closing part - Hazenberg
- 17.00-17.45** **Refreshing drinks and informal discussion** (*'Fonteinpatio'*)

