研究成果の刊行に関する一覧表レイアウト

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1. **シスチノーシス（シスチン蓄積症）診療ガイドライン**2018
   監修：厚生労働省難治性疾患等政策研究事業　ライソゾーム病（ファブリー病を含む）に関する調査研究班
   編集：シスチノーシス（シスチン蓄積症）診療ガイドライン
   診断と治療社
2. **ゴーシェ病**UpDate
   責任編集：衞藤義勝、井田博幸
   編集（50音順）：大橋十也、奥山虎之、酒井規夫、高柳正樹、成田綾、難波栄二
   診断と治療社
3. **週刊　医学のあゆみ**　Vol.264 No.9 2018 3/3
   第一土用特集　ライソゾーム病のすべて　企画：衞藤義勝
   医歯薬出版株式会社

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２７．矢部普正

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【付録】

付録１.

主催：厚生労働省　難治性疾患等政策研究事業 ライソゾーム病（ファブリー病含む）に関する調査研究班

第4回市民公開フォーラム

日　時：平成30年1月14日（日）13：00～18：00

会　場：東京慈恵会医科大学　大学1号館3階講堂

■プログラム■　総合司会：小林博司（東京慈恵会医科大学）

13:00-13:05　班長挨拶
衛藤義勝（班長・東京慈恵会医科大学）

参加費 無料（どなたでも参加できます）

13:05-13:30　基調講演
司会　衛藤義勝（東京慈恵会医科大学）
我が国の難病対策の現状と今後の展望（仮題）
厚労省難病対策課

13:30-14:30　I．ライソゾーム病の治療の進歩
司会　酒井規夫（大阪大学）、加藤剛二（名古屋第一赤十字病院）
1）骨髄移植治療の進歩ー　矢部普正（東海大学）
2）中枢神経系治療の進歩　奥山虎之（国立成育医療センター）
3）ライソゾーム病遺伝子治療の進歩　大橋十也（東京慈恵会医科大学）

14:30-15:30　II．診断ガイドライン
司会　石垣景子（東京女子医科大学）、福田冬季子（浜松医科大学）
1）ファブリー病　小林正久（東京慈恵会医科大学）
2）ゴーシェ病　成田　綾（鳥取大学）
3）ムコ多糖症Ⅰ型　小須賀基通（国立成育医療センター）
4）シスチン症（シスチノーシス）　高柳正樹（帝京平成大学）

15:30-16:00　III．ライソゾーム病のトランジション問題ー
司会　今中常雄（広島国際大学）
1）医師の立場から　高柳正樹（帝京平成大学）
2）患者会の立場から　原田久生（全国ファブリー病患者と家族の会）

16:00-16:10　休憩

16:10-17:50　IV．ライソゾーム病トランジション問題を含めた拠点病院構想パネル討論
司会　辻　省次（東京大学）、原田久生（全国ファブリー病患者と家族の会）
1）概　要　高橋　勉（獨協大学）
2）拠点病院構想の患者会からの要望
①重篤患者の拠点病院　本間リス（NPO　ALDの未来を考える会）
②ポンペ病の拠点病院　岡嶋俊文（NPO　全国ポンペ病患者と家族の会）
③ムコ多糖症拠点病院　吉井一能（日本ムコ多糖症患者家族の会）
④ゴーシェ病拠点病院　古賀晃弘（日本ゴーシェ病の会）
⑤MLDの拠点病院　吉崎容浩（異染性白質ジストロフィー患者会）
⑥ファブリー病拠点病院　原田久生（全国ファブリー病患者と家族の会）
全体討論ー患者会メンバーと医師と厚労省でのパネル討論

17:50-18:00　班長挨拶
衛藤義勝（班長・東京慈恵会医科大学）

事務局：（財）脳神経疾患研究所附属 先端医療研究センター＆遺伝病治療研究所
（新百合ヶ丘総合病院内）　Tel:044-322-0654

付録２.

The 8th International Collaborative Forum of Human Gene Therapy for Genetic Diseases

第8回 国際協力遺伝病遺伝子治療フォーラム

【会　場】東京慈恵会医科大学大学1号館3階講堂（〒105-8461　東京都港区西新橋3-25-8）
【日　時】2018年1月18日（木）10:00-18:00
【参加費】3,000円
【当番幹事】奥山　虎之（国立成育医療研究センター）

テーマ：「先天代謝異常症の遺伝子治療臨床研究」

開会の辞・ご挨拶
第8回フォーラム当番幹事：奥山　虎之（国立成育医療研究センター）
国際協力遺伝病遺伝子治療フォーラム実行委員長：衛藤　義勝（国立成育医療研究センター）

シンポジウム1．わが国の遺伝病遺伝子治療臨床研究の進歩
Gradual Improvements in the motor and cognitive function after gene therapy for patients with AADC deficiency：小島　華林（自治医科大学小児科）
Stem cell gene therapy for primary immune deficiencies in Japan：小野寺雅史（国立成育医療研究センター）

シンポジウム2．国内企業による遺伝子治療研究の現状
Department and Application of Stealth RNA Vector：中西　真人（株式会社ビバイオ、産業技術総合研究所）
HGF plasmid gene therapy for the treatment of critical limb ischemia：山田　英（アンジェス株式会社）

企業セミナー
TaKaRa Bio

シンポジウム3．海外企業による遺伝病遺伝子治療の臨床開発
bluebird bio, Inc.（Dr. Gary Fortin）：Interim results from a Phase 2/3 Study of the Efficacy and Safety of Ex Vivo Lenti-D™ Gene Therapy for the Treatment of Cerebral Adrenoleukodystrophy
Orchard Therapeutics（Dr. Jesus Garcia-Segovia）：Primary Immunodeficiency（Tentative）
Spark Therapeutics（Dr. Daniel C. Chung）：Investigational Gene Therapy for RPE65-Mediated Inherited Retinal Disease
Pfizer Inc.（Dr. Toru J. Seo）：Gene Therapy to Drive Transformative Medicine for Intractable Diseases –Pfizer's Prospects on Approaches and Challenges Ahead-

シンポジウム4．先天代謝異常症に対する遺伝子治療
Novel therapeutic approach for the treatment of inherited and metabolic diseases：奥山　虎之（国立成育医療研究センター）
Current status of gene therapy for inborn error of metabolism：大橋　十也（東京慈恵会医科大学遺伝子治療研究部）
Gene Therapy for Methylmalonic Acidemia（MMA）and Related Disorders；Lessons from Patients and Mice：Dr. Charles P. Venditti（National Institutes in Health）
Strategies for Effectively Treating Complex Lysosomal Strage Diseases：Dr. Mark Sands（Washington University in St. Louis）
CNS targeted ex vivo gene therapy for mucopolysaccharidoses（Tentative）：Dr. Simon Jones（Manchester University Hospital）

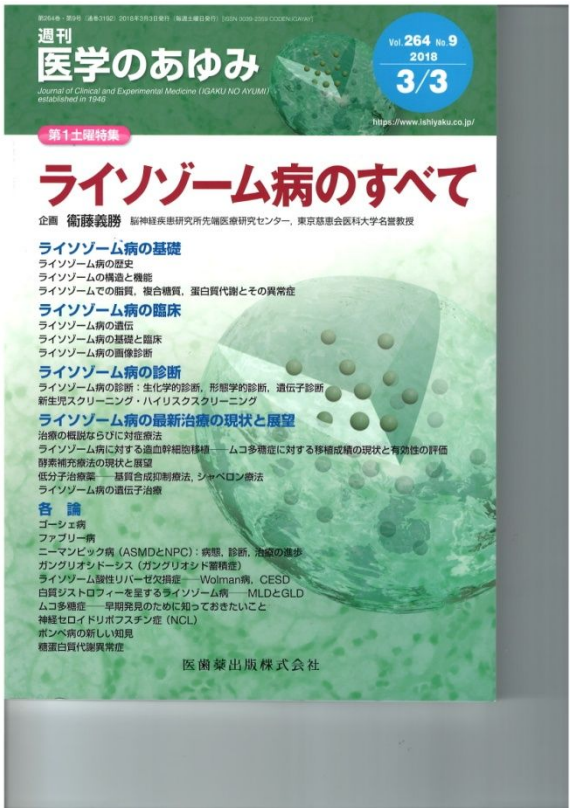
特別講演
ライソゾーム病の遺伝子治療（仮題）：Dr. Chester Whitely（University of Minnesota）

閉会の辞・ご挨拶
日本遺伝子治療学会理事長：金田　安史（大阪大学）
第9回フォーラム当番幹事：大橋　十也（東京慈恵会医科大学遺伝子治療研究部）

（問い合わせ先）国立成育医療研究センター　遺伝診療科　小須賀基通
電話：03-3416-0181、FAX: 03-3416-2222、Email: kosuga-mo@ncchd.go.jp

主催：国際協力遺伝病遺伝子治療フォーラム実行委員会
後援：国立研究開発法人日本医療研究開発機構（AMED）「医療技術実用化総合研究事業（代表：奥山虎之）」
日本医療研究開発機構（AMED）委託費「難治性疾患実用化研究事業」
「医薬品等規制調和・評価研究事業」（研究開発代表者：小野寺雅史）
厚生労働省難治性疾患等政策研究事業（難治性疾患政策研究事業）（研究班代表者：奥山虎之）

付録３.



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第1土曜特集

ライソゾーム病のすべて

企画　衛藤義勝　脳神経疾患研究所先端医療研究センター，東京慈恵会医科大学名誉教授

ライソゾーム病の基礎
ライソゾーム病の歴史
ライソゾームの構造と機能
ライソゾームでの脂質，複合糖質，蛋白質代謝とその異常症

ライソゾーム病の臨床
ライソゾーム病の遺伝
ライソゾーム病の基礎と臨床
ライソゾーム病の画像診断

ライソゾーム病の診断
ライソゾーム病の診断：生化学的診断，形態学的診断，遺伝子診断
新生児スクリーニング・ハイリスクスクリーニング

ライソゾーム病の最新治療の現状と展望
治療の概説ならびに対症療法
ライソゾーム病に対する造血幹細胞移植――ムコ多糖症に対する移植成績の現状と有効性の評価
酵素補充療法の現状と展望
低分子治療薬――基質合成抑制療法，シャペロン療法
ライソゾーム病の遺伝子治療

各　論
ゴーシェ病
ファブリー病
ニーマンピック病（ASMDとNPC）：病態，診断，治療の進歩
ガングリオシドーシス（ガングリオシド蓄積症）
ライソゾーム酸性リパーゼ欠損症――Wolman病，CESD
白質ジストロフィーを呈するライソゾーム病――MLDとGLD
ムコ多糖症――早期発見のために知っておきたいこと
神経セロイドリポフスチン症（NCL）
ポンペ病の新しい知見
糖蛋白質代謝異常症

医歯薬出版株式会社

付録４.

Practical guideline for the management of cystinosis 2018

シスチノーシス（シスチン蓄積症）診療ガイドライン2018

監修
厚生労働省難治性疾患等政策研究事業
ライソゾーム病（ファブリー病を含む）に関する調査研究班

編集
シスチノーシス（シスチン蓄積症）
診療ガイドライン作成委員会

診断と治療社